# PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

## BOARD OF GOVERNORS MEETING

December 7, 2015

Mayflower Hotel
1127 Connecticut Ave, NW
Washington, DC 20036

[Transcribed from PCORI teleconference.]

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AGENDA

AGENDA [Continued]

P R O C E E D I N G S

[10:15 a.m.]

OPERATOR: Dr. Norquist, the floor is yours.

CHAIRMAN NORQUIST: Thanks. Good morning. I'm Dr. Gray Norquist, chair of the PCORI Board of Governors and I want to welcome you to today's Board meeting, which is being held in Washington D.C. in person. It's also being held by video conference -- I mean, excuse me -- teleconference and webinar. For those unable to attend in person, instructions for logging in or calling in are available on our website at pcori.org/events.

All Board members are present in person, except Francis Collins who has designated Mike Lauer in his place. I want to remind everyone that disclosure of conflicts of interest of members of the Board are publicly available on our website and are required to be updated annually.

Members of the Board are also reminded to update your conflict of interest disclosures if the information has changed. You can do this by

contacting your staff representative. If the Board will deliberate or take action on a matter that presents a conflict of interest for you, please inform me so that we can discuss how to address the issue. If you have questions about conflict of interest disclosures or recusal relating to others, please contact your staff representatives.

All materials presented to the Board for consideration today will be available during the webinar and then after will be posted on our website at pcori.org. The webinar is being recorded and will be posted by the end of the week. We have a scheduled public comment period today from 5 until 5:30 p.m. EST. If you are interested in registering to provide public comment, please visit our event page for instructions. Alternatively, you can always e-mail us at info@pcori.org or provide input through our website.

Finally, a reminder. We're live-tweeting today's activities on Twitter. Join the conversation at #PCORI.

And the first item is an approval of the minutes. And so, that's from our November 17th Board meeting. So, I need a motion to approve the minutes.

VICE CHAIRMAN BARNETT: Moved.

CHAIRMAN NORQUIST: Kerry? Thank you. And a second?

DR. LEVINE: Second.

CHAIRMAN NORQUIST: Thanks, Sharon. Any discussion about the minutes? Any comments? Changes?

[No discussion.]

CHAIRMAN NORQUIST: Okay. I think we can just do a voice vote here. So, all in favor?

[Chorus of ayes.]

CHAIRMAN NORQUIST: Anyone opposed?

[No response.]

CHAIRMAN NORQUIST: And, anyone abstaining?

[No response.]

CHAIRMAN NORQUIST: Okay. That takes care of that one. And so, Joe?

DR. SELBY: Good morning.

CHAIRMAN NORQUIST: Joe Selby is our executive director.

DR. SELBY: Good morning, Gray, and good morning, staff members, and public who are here with us and listening online.

We're going to go out of order this morning because of some time constraints on presenters. At our September Board meeting, the Board of Governors asked us specifically about patient engagement and stakeholder engagement in PCORnet, our very large infrastructure project. And in response, I've invited Sue Sheridan, who is the director of patient engagement at PCORI and has been with us for four years and has been trying to help researchers understand and work with and actually learn on behalf of PCORI as well what valuable, valid, useful engagement looks like -- to present to us.

And also, Sharon Terry, who is -- get ready, three titles here. She is the principle investigator of one of the patient-powered research

networks in PCORnet. Sharon is also a co-principle investigator of PCORnet's coordinates coordinating center, and her responsibilities are directed towards coordinating all of the PPRNs and coordinating the work and supporting the PPRNs in creating their own networks and networking among themselves and in playing crucial, central roles in PCORnet as a whole.

And a third title is a result of governance changes at PCORnet as we enter Phase 2. We now have three central strategy committees of the PCORnet Council and one of them is the engagement committee, and Sharon was elected by her peers within the PCORnet Council to chair the engagement committee.

So, thank you both for being here this morning. And I think we will start with Sue and hear about engagement in PCORnet from the PCORI perspective and then hear from Sharon about engagement in PCORnet from inside, from on the ground inside PCORnet.

Thanks, and I'll turn it over to you, Sue.

MS. SHERIDAN: Great, thank you, Joe.

This presentation really is intended to share with you and to demonstrate the real synergy and overlap between the strategic plan for engagement for PCORI and the engagement that Sharon's going to talk about in PCORnet. And we're going to share -- I'm going to demonstrate how we're discovering the opportunity to really share in leverage, our respective tools, and resources that we're creating in PCORI and PCORnet that we think is mutually-beneficial to each other.

And then, Sharon's going to really demonstrate and she's going to share the engagement plan for, say, two of PCORnet. So we're going to begin by sharing what really makes PCORnet different -- and PCORI -- different. And that really is the engagement as the tool for transformation.

And so, this graphic just illustrates that it's engagement that makes -- that really separates PCORI in the world of research and PCORnet in the world of a development of a data research network.

And in PCORI, the engagement really is to both at the enterprise level and at the individual project level, where patient and stakeholder engagement is required. It really serves to influence that the research is patient-centered, that it's relevant and useful to establish trust, and to encourage successful uptake of the research findings.

In PCORnet -- and Sharon will talk more about this -- the engagement at both the enterprise and the individual PPRN and CDRN level really is the cornerstone in building trustworthy patient-centered research network.

So, this next slide just demonstrates -- you'll recognize this. This is from our original strategic plan that we built all together back in 2013. It demonstrates that PCORnet is aligned with the principles and the thoughts that we had when we created the plan for engagement to develop community skills and PCORI -- that was our number one goal. To successfully establish an infrastructure for patients, caregivers, and other stakeholders to increase information, engage in

research dissemination and evaluation, and to engage that community in the research process, and to promote dissemination and implementation. And Sharon is going to walk through what they're doing that follows right into those long-term goals.

The next slide really shows that there really is significant synergy between PCORI's engagement, core principles, and strategies, and that of PCORnet. And you can see kind of in this overlap zone that both PCORI and PCORnet are striving to build a community that is skilled in PCOR. That we're both creating repositories of models and promising engagement practices to share with each other and the broader research community. We're both creating tools and strategies to ensure meaningful engagement in research. That is really one of our legacies, I think, at PCORI and PCORnet, and that we're building robust measures of engagement in both initiatives.

So, I'm just going to show you a couple slides on some of the tools and frameworks that PCORI has built with the patient community and

stakeholder community that are available to PCORnet. And we've talked about doing some modifications with some of these tools to really fit with PCORnet. But we have engagement rubric -- now, all of these are in your appendix if you want to reference any of these tools that are created. We created a patient engagement framework for infrastructure development that was used at the beginning of Phase 1 of PCORnet.

We've also created a compensation framework. This is the compensation framework for patient partners in research. This was vetted by patients, created in partnership with the Patient Engagement Advisory Panel. This is now online and referenced by several organizations. We also have tools and strategies to evaluate engagement. We have examples of metrics and measures that we are eager to work together on how to develop that together.

And then, we have just some training capacity-building tools and programs to help build this community that both PCORnet and PCORI are

building. One of the main ones is the Eugene Washington Engagement Awards. I'm going to give you a couple examples. And then, we are in the process of developing a PCOR/CER training. We have our ambassadors. We actually have PCORnet and PPRN -- especially PPRN -- well, actually some CDRN -- folks now have become PCORI ambassadors. So, there's a lot of nice cross-fertilization.

We're in the process of creating a Team Science curriculum, and we are doing -- in the process of creating rubric training video for everybody undertaking research and engaged research.

Just a couple examples. You have several examples in your appendix, but just to highlight. The Engagement Awards, that's led by Lia Hotchkiss, that's here if you have any questions -- back there. This is just to demonstrate that we're creating tools and we have resources in PCORI that really serves as a resource to PCORnet and the broader research community. This is one that's called Better Said. This is actually an engagement

award that is really unifying CDRNs and PPRNs and helping them work together on going forward to prepare arthroplasty patients and other stakeholders to participate collaboratively in patient-centered CER.

Another one of our engagement awards that not -- the awardee is not a PPRN or CDRN -- this was to the Reagan-Udall Foundation. This is called Big Data for patients. This is just the development of a curriculum that's going to help patients understand what is big data. What are the benefits? What do they need to be aware of? And so, this is again broadening the knowledge of our patients and our patient community about big data, which will, in effect, support the efforts of PCORnet.

So at that, I'm going to pass it on to Sharon so she can share how PCORnet and Phase 2 -- as they are becoming more independent -- that we would have resources at PCORI, but as they take on PCORnet Phase 2, she's going to share that plan that they're in the process of developing.

MS. TERRY: Great. Thanks, Sue. And thanks very much for being here with all of you. I think Joe went over how I'm related to PCORnet.

So, the engagement goal for PCORnet is essentially to create a truly participant -- and we use these two words interchangeably -- participant and patient-centered research network by systematizing -- which is a very important word to us, that we're not just one-offing these things -- overall engagement, governance, leadership, et cetera -- research practices to implement strong process for measurable engagement strategies and ensure that all of our activities include everyone in terms of the participant types.

We'll execute this on two levels; the PCORnet enterprise level, so that's across PCORnet as a network, and then the individual networks focusing on engagement in several stakeholder types. Not limited to these, but specifically patient participants, clinical provider investigative researcher, and community and systems leaders.

We had a work group for the last quite a few months, five or six months. I led that with Rachael Fleurence. Sue was part of it. Bray Patrick-Lake, who I think many of you know from the coordinating center at Duke -- and then several people from each PPRN and CDRN has participated as well. We also had a very robust and broad process that engaged all of the stakeholders.

So, the Engagement Committee that is now mandated -- so, Joe mentioned we have a Data Committee, a Research Committee, and an Engagement Committee to be sort of the three legs of the stool that is PCORnet. The Engagement Committee has decided that all engagement strategies, activities, and products be monitored by the Executive Committee and the PCORnet Council -- and to remind you, those terms -- Executive Committee are two PPRNs, two CDRN leaders, the coordinating center, and PCORI working together as sort of an executive board. The Council is being one representative of each of the PPRNs and CDRNs that are now funded.

Our observations were many, and I'll be

very brief with these. They're available for you to look at. Essentially, that we recognize that -- we've begun to recognize that patient-led research in the form of the PPRN needs more, and we've begun to talk about that. Engagement is valuable and must assess needs. We have to start to understand experience, utilizing tools that would give us better access to services, as well as amplify the voices of the participants in the process.

One of the things we know about engagement is that you cannot expect people to just enroll to be used by the research system. There must be value given back to those individuals, it must be a virtuous cycle, and we're looking for what's the magic sauce of those cycles.

Engagement requires deep and authentic interactions. Again, you can't just tell people "this is engagement". Engagement is relationship, and we all know how difficult and how exciting relationships are. They are not transactional, and so we're really trying to get to what is the coordination and systemization of such activities.

They must be stakeholder-driven. These cannot be done top down. However, we believe they must be assisted centrally, i.e. a coordinating center, but conducted throughout the network in a federated model. So, if some networks are better at some things and others at others, we should be using those networks in a federated sense of imparting that knowledge and those tools.

And then, PCORnet has the opportunity to improve population health through meaningful stakeholder engagement addressing factors that impact health outcomes for the individual through interpersonal and community levels as well.

So, we looked at the strategic activities that were, so far, being undertaken and this was, again, a four-, five-, and six-month process. Analysis of all the engagement products, tools, and activities both within and without PCORI -- so, we looked at what was happening in PCORI and then beyond. A framework for engagement activities over the next three years of Phase 2 is what we mapped. We looked at development of criteria and measures

for engagement.

Some exist, but they're somewhat nascent. They need more work. We need to implement and improve process for systemic engagements for energies throughout all the levels I talked about. We need to recommend engagement tactics within PCORnet. Identify a process for ongoing coordination with PCORI to ensure that all the great stuff that's coming out of the EAIN awards -- the other activities that Sue is working on -- are really coordinated well.

And then, to outline a process for routine sharing within the network, and we're looking at things like robust commons, the typical ways of sharing information, but also some novel and interesting ways as well.

And this is the last slide I have. We put together something called a Key Driver Diagram that essentially says if our goal is to create this authentic participant-centered network that's systematizing engagement and measuring it. What are the drivers for that? And we list here some

drivers. Again, I'm not going to go through all of this. And then, what are the interventions we need to get those drivers done?

So, the committee is actually working on the intervention level now to start to put those together. We actually pick committee members in the next month or so. All the PPRNs and CDRNs have nominated individuals to the three committees. We got an enormous number of nominations, it's fabulous, and we'll put together the committee that will actually work on these interventions to get to the drivers, to get to the goal, have dashboards that are transparent so we can see our progress, where we need to improve, et cetera. And so, we're pretty excited about this activity going forward, particularly that we systematize and measure things so that they are replicable and transformable.

One of the projects that we're undertaking rather soon -- January 20th, 19th and 20th -- is a trustworthiness meeting. We began by looking at public trust and said when we say that it sounds like we want the public to trust us. And in fact,

we should be asking how is it that we are trustworthy? Because when we ask the other we're putting the onus on the public. So instead of that we're putting the onus on ourselves. We're going to look at how do we describe the characteristics of trustworthy engagement? How do we examine successes and failures and building trustworthy research initiatives, including things like care data and other things that have tried to do this and failed.

Beginning a robust dialogue about trustworthiness within PCORnet that's open and honest and frank. Creating recommendations for PCORnet and its stakeholders and then creating recommendations for the networks. Again, we're not going to ask how do we get people to trust us, but instead how are we trustworthy? The onus is really on us.

Thank you.

CHAIRMAN NORQUIST: So, thanks. We'll open it up now for questions or comments. If you'd put your tent card up and then we'd go around.

Barbara is the first hand I saw so far.

DR. McNEIL: That was a lovely presentation. My questions will describe a little ignorance.

So the first question is to Sue and then the second one is to Sharon. And the question to Sue is, you mentioned you had some training awards in one of your early slides. How are you going to measure the success of those training awards and what is the outcome?

MS. SHERIDAN: Right now we are in the process -- all of our training awards are underway, so we haven't had any outcomes yet.

DR. McNEIL: What will they be?

MS. SHERIDAN: We will have a variety -- we can invite Lia to share some of the -- we've got over 70-some awards in the Eugene Washington Engagement Awards. Many of them if not most of them are training and development. So they're training curriculum in helping communities, mostly of patients and other stakeholders, understand CER and PCOR and how they can engage more robustly in

that. So it could be a training award to a patient organization.

DR. McNEIL: Got it, so there were no outcomes associated with those training awards?

MS. SHERIDAN: The outcomes are actually -- they're deliverables in that they deliver those training curriculums -- the products to us.

DR. McNEIL: So then question for Sue and this is the one -- I'm sorry and I didn't quite get it this morning and I don't quite get it now. When you talk about your incredibly comprehensive set of activities to get patients involved in this, the question that I have is, I guess two questions. One is, this is an incredibly long list of process items. When ultimately at the end of the day you want to have patients voluntarily sign up for activities [inaudible]. That's really your goal. [Inaudible.]

So then the question is one, when do you think that will happen and two, and this is the real critical one, what makes you think that your activity will be more successful getting patients

[inaudible] study of cemented versus cementless prosthesis hip replacement than having the orthopedic surgeon pull in patients?

I'm just trying to get a sense of where your marginal contribution is.

MS. SHERIDAN: Yes, I hope it's not going to be marginal. I hope it's going to be big.

DR. McNEIL: I'm sorry. Did you hear me? Should I do it again? Okay, so what I was questioning Sharon was that all of what you mentioned is really process items. And my first question was, how soon are you -- how close are you to translating those process activities to a real outcome involving the engagement of patients and real comparative effectiveness of the research.

And the second one was, how do you know or will you know or do you know how much more successful you are going to be in getting your patients in your various networks to enroll in, say, cemented versus cementless arthroplasty versus a trial run by orthopedists. And I use the term "marginal" in not a derogatory sense but in a true

analytic sense. What is your extra contribution to that enrollment effort?

So, how are you going to know that and when will you know it?

MS. TERRY: So, one of the things I did not present for sake of brevity is that we have a whole host of measures that are actually concrete metrics around not only enrollment -- because I want to say, again, that we do not view engagement as simply a way to increase the numbers of people that are recruited. I think that's a kind of baseline. And then I think we're really looking at how do people become engaged and asks the questions that are meaningful to them?

So, cement or cementless may not be a critical question to them, or at least one that they yet understand. And so, there are several stages toward that kind of awareness and growth understanding. What we're looking for is an engaged public that then is ready to be engaged in these various projects. And whether or not the better path is to have the orthopedists enroll them

versus robust patient engagement process is going to be part of what we measure.

I would also say that some things will be done in a traditional manner and we have to say to ourselves, are we happy with the 3, 4, 5, and 6 percent enrollment rates that we currently have. And if we are, then I think that's great in that particular -- if that's all we need in a particular area, then that's super.

In most cases for CER I think we're really saying, what are the important questions for patients? How do we get them to understand how they can be part of the process of explaining those? And do more than just sign up as a metric of their engagement.

I don't know what those measures are yet because that is part of the work of the committee is early on to establish the measures that will give us some sense of that.

And the last thing I'll say is, for PCORI/PCORnet overall -- and I am in the Co-PI position actually working on both CDRNs and PPRNs

for engagement, network, and commons, we are looking at what are the dashboards that we need to have that do measure engagement, participation, true relationship, and the growth of participation in a meaningful way. And we are beginning to have some of those metrics but we don't yet have those nailed.

CHAIRMAN NORQUIST: Allen.

DR. DOUMA: Thank you both very much. As you know, I in particular but this whole Board is really strongly engaged in engagement. We think it's critically important.

One of the challenges that many of us face is when talking to the outside world about, so you do engagement, so what? Can you talk about PCORI's research strategy, overall strategy, and finding how we're going to measure the impact of engagement on CER and even mores on better health outcomes? What is that plan that we have in place at this point in time?

MS. SHERIDAN: Okay, Lori Frank and Laura Forsythe have offered us ongoing presentations on

engagement in CER at PCORI, but I can share that right now what we're seeing is, you know, with the impact at various touch points along the way in the process of research in terms of how many patients and stakeholders are engaged in determining the research question. How many are engaged in developing and determining comparators and eligibility criteria -- so we're tracking that right now. We're not at the end yet because as you know, our research is still underway.

But, we are tracking it and there -- and I think you've heard some of these. There are some outstanding presentations that are showing us right now the impact of engagement in the process of research. And something we're having ongoing conversations about how we're going to track this, for example -- something that we're currently talking about is are we developing trust through this process by engaging our patients and our stakeholders and will that help feed the implementation once we have those outcomes?

So, that's where we are right now. And in

terms of measuring engagement to our re-enact -- and then in our engagement activities, we have a whole set of different measures and metrics.

DR. DOUMA: I would just suggest that we have a more defined research protocol or research strategy with regard to when we do get outcomes. And in particular, what outcomes are we looking at and various research that we're now funding will be most fruitful in determining the impact of engagement.

I don't think we ought to wait until we have outcomes before we decide how we're going to get the research done.

CHAIRMAN NORQUIST: Okay, I don't see any other tent cards. Let me make a point and then I'll make a question.

Sharon, I thought perhaps what you would say about recruitment -- it's not just about the numbers, it's about the type of patients that you get. I think that one of the key things that could be different is a broader representation. Because, sure, orthopedic surgeons might get you but the

type of patients you get might be very specific and homogeneous, if you will. So I think one of the opportunities here is not just the numbers but the broad representation of patients.

So, that leads to my second point and kind of question, which is as someone who has worked a lot with under-represented populations, often have been taken advantage of. And so, I wonder how you're going to address this issue of the certainty that the people who are the participants that you engage really feel an ownership and a protection, if you will, on how that's being dealt with. Because that's really critical. If this is going to survive and really be a new way of doing business and getting broader populations, that really has to be attended to.

MS. TERRY: So for PCORnet, one of our kickoff activities is this trustworthiness meeting in which the two days, the speakers are the people who had come from the communities who have been through the exact kinds of things you've described. So, the family of Henrietta's Lacks, Ruha Benjamin

who is known for her work in disparities and engagement, other individuals, the Havasu Tribe, et cetera will be talking to us about here's what you need to be doing to enable a relationship between you and the communities you need to work in. We cannot come from our top-down positions and expect to helicopter in, as we well know, and instead I think we really need to be creating relationships in the very communities that need to be empowered to not only just be invited to the table, but for them to be starting to set those tables.

MS. SHERIDAN: And I would like to just add to that what we're doing in PCORI. That again, I think we're going to complement each other as we grow in this direction. That we've got some engagement awards that are specifically to some of the underserved communities where we really want to build trust. We have Pastors 4 PCOR in Chicago, and we have others that are really bringing in communities to help them understand their opportunity in research. So again, we're developing tools, our engagement officers are

collecting best practices and our PCORI portfolio on this very issue that we want to share with PCORnet and vice versa.

MS. TERRY: And also, Gray, two of the new PPRN, at least, are extremely good at this. One is PrideNET which is about 150 gay, lesbian, bisexual, queer, transgender individuals across the United States in coalitions. They're working across many, many communities including the homeless, et cetera. And then another CPPRN, which is a community PPRN that's done excellent work with homeless individuals and has an amazing rate of 97 percent participation rate of homeless and indigent people in the projects that they run. So, we're hoping -- and we are already -- learning a great deal from them.

CHAIRMAN NORQUIST: Great, thanks. So, we look forward to seeing that. Harlan Krumholz.

DR. KRUMHOLZ: Yes, I just wanted to commend the comments and presentations. The words the Chairman just articulated I think are so important -- and Sue as well -- but I know Sharon

has been pushing these concepts to citizen science for a long time.

I want to just say that the notion of partnership -- these are test-able. Barbara, you were raising this question of whether we should test some of these ideas, but there's also just a sense of what's right, you know? In terms of the respect and honoring of people who are involved in the research. The understanding that they're bringing to the table things of importance.

And I agree with you, Gray, it's about who. But it's also about what it feels like to be in these studies if you are expecting to be part of the team, you are expecting to hear what the results are at the end. You're expecting not just collecting data from, but actually being told you're part of an important quest to try and generate that knowledge.

I do think we need to be collecting data about this because in the end, these are complex interventions. And even though we can check the box, we can be doing it poorly or be missing an

active ingredient that's very important.

And so, I'm firmly committed to the course but I do believe as a learning organization this is an important piece of this. Both of you got into this because of personal experiences and have contributed so much to the world, turned something that was very challenging into something that benefited many other people. There's good in that, and we need to be able to foster that and create those opportunities. But we also need to figure out how we can do it in a smart way. And I think the notion for this Board should be about how we can support the meta-studies around -- so we're supporting the studies in the middle but we should also be funding some studies that are studying the way which we're conducting the work.

I know Mike has already done good work about studying the peer review process, for example. Those are the kind of things that I hope will come out of PCORI that should be there. In the course of doing this we should be building studies of independent people, not those of us who

are sold and bought and believe in it so deeply, but people who may even have an independent nature to them so that we can get really fair assessments of these things. And I think our investment there could also generate a lot of -- a good legacy for PCORI. It's not -- we've learned something negative. We're not going to stir off the notion that this is the right thing to do, but maybe we can do better. I think that's going to be an important part of our work.

CHAIRMAN NORQUIST: Barbara.

DR. McNEIL: I agree with you, Harlan. And I wonder if that is the case, and I think it should be the case. You're amplifying on this comment suggestion, what is the value of this and how do we prove it? Because if we can't prove it, it's really not going to last, no matter how good it is and how honorable and how right it is to do. People are going to ask.

So I wonder if when you're thinking about your engagements and your areas with your various groups, you might think about one particular

disease or syndrome or whatever. You talked about some cross-disease items at breakfast this morning.

Okay, suppose we got a group together to discuss hip arthroplasties or atrial fibrillation or pain or whatever. What exactly would we be measuring and how would we look to see if it led to an expanded network, as Harlan suggested? And really drive to doing that.

MS. TERRY: Great suggestion, Barbara. So, we did about maybe -- I don't know, six weeks ago -- polled the CDRNs and PPRNs around those very questions. Within the disease context, what are the questions that you've stood up to answer and how are you going about answering those? To start to look at what are the synergies across our small, 100 organizations, 62 PIs, PCORnet. And we are discovering amazing synergies within disease areas that I think will lend a kind of robustness to building the networks that Harlan was talking about.

CHAIRMAN NORQUIST: Bob Zwolak.

DR. ZWOLAK: Thank you. It was a very

nice presentation. I'd obviously buy into the huge importance of engagement.

My question has to do with types of disorders and age, because I have the sense there must be an enormous variation in the likelihood of succeeding in the engagement realm based on the age and disease burden of the people you encounter. In my -- every day, I see people who are 70s, 80s -- it's not unusual for 80 to be my median age in clinic, and those people basically just want to know what they have to do to get better.

And when I sit there thinking about my role in PCORI and then I go to clinic and I see these poor people dragging in I say, okay, how are we going to energize -- who do we engage? What's the secret sauce to some of these really difficult populations?

MS. TERRY: So I'll answer and I'm sure Sue has an answer as well.

My mom died last week of Alzheimer's disease, so I have an acute sense of what it means for some of these conditions where we're dealing

with either elderly population, people with dementia, people with just a fatigue that doesn't really allow anything else. So, we can't say, okay, engagement for you is you should tweet twice a day and, you know, that we really have to be very realistic.

So, I think we should look at caregivers very much. I think we should stop thinking about just the pro band, and the families, the communities, the neighbors are also who we're talking about when we say "participants" and when we say "engagement".

I think we also need to say, let's not burden people further, but also let's not be paternalistic. So for me to say all 80-year olds should not be engaged would disregard my mother-in-law who is 85 and wants to be engaged in her own healthcare and is frustrated at a recent experience with breast cancer.

So, let's gauge things with people where they live, where they are -- which we actually need to do for most populations. And then particularly

when we come to people who are decisionally-impaired in some way, to find ways to engage the community around them in a respectful way. But we are not talking about putting more burden either -- the other piece I think we absolutely have to talk about as a clinician that we often forget the clinician in our conversations, and we really need to think about how do we support the clinician in engagement as well.

MS. SHERIDAN: And just to add to that. Sharon said it beautifully, in that we see several of our PCORI projects around the elderly -- our falls project that we have in improving healthcare systems, one that's keeping patients or the elderly at home rather than going into long-term care. So, we're seeing engaged patients, consumers in their 80s. We have some patient partners in their 80s that are involved in PCORI.

So, I think the word is opportunity. The opportunity is there, the invitation is there. We work with several patient organizations and caregiver organizations, like Sharon said, to offer

that opportunity. And if people 80s, 90s want to be engaged, they're engaged. If their children are engaged, their caregivers -- so, that opportunity is always open at PCORI.

CHAIRMAN NORQUIST: Well, thank you both very much. That was a wonderful presentation. I'm sure we'll be hearing more about the outcomes later. So, thank you.

Sue, would you bring the thing back here? Thanks. The slide control.

So, the next item is the Executive Director's Report and the End-of-Year Dashboard review. So, Joe Selby, our executive director, will do that session.

DR. SELBY: Thanks, Gray. And this first director's report is going to be very brief. There are two pictures that I'd like to share with you. Each one of them has affected me greatly in the last few weeks to months, and affected other staff as well. They're both big ideas and big thoughts that we need your involvement with.

So, here's the first one. Oh, I showed

you both at the same time. That was a mistake. Here's the first one. This is just a chart of our revenues to the PCOR Trust Fund in green and our commitments throughout the first five years of our existence. So, five years is a little bit of a stretch because we weren't able to begin funding until just -- until May of 2012. But in that time frame we committed $1.2 billion. We now have -- we're more than halfway. This is kind of part of the message. We are more than halfway through the Congressionally-approved lifespan of the first part of PCORI.

We have committed about half of our resources -- and actually, if you look at that $1.3 billion you can subtract out over $100 million for the four targeted PFAs that you approved I think at the September Board meeting, in the last couple months -- actually, it was before the September Board meeting. Those PFAs on the use of opioids, on the use of novel oral anticoagulants, on the treatment of severe depression, and on multiple sclerosis have been posted and letters of intent

submitted. So, you might reduce that to $1.2 billion. So, there's two ways to look at it.

We still have a lot of committing left to go, and on the other hand, it is a finite amount of money. If you think about how much we have funded in the term of -- in terms of the targeted funding announcements, the pragmatic clinical studies, and the broad awards, that's a finite number of projects, and that means that we've got a limited number of projects left to fund.

And that just simply is background information for ongoing discussions about how we make the best commitments. About how we ensure that the research that we attract, solicit, and ultimately fund is as relevant to the needs of patients and other stakeholders as it can be, and as exemplary as it can be of what an organization like PCORI is interested in and does fund.

So, you have to hold those two thoughts in your mind at one time. We have a lot of commitments to make and those commitments are limited. You also see the tapering of the blue

bars. It is a lot more. We decided this early on that we don't want to be committing a lot of money in 2019. We'd prefer to have it more in place earlier. So, that's another thing to keep in mind.

And I know that the SOC will be thinking about this. It always has, it always will. But I think that it's been energized by looking at pictures like this to involve themselves even more deeply in making sure that we're doing everything we can to get to meaningful research in as timely a way as we can.

So, that's the first thought.

DR. DOUMA: Joe?

DR. SELBY: Yes.

DR. DOUMA: Can we ask questions as you go along so you don't have to jump back?

CHAIRMAN NORQUIST: Do you want to make this point before you ask the question, or do you want to let him ask --

DR. SELBY: No. This is a different point. So, sure.

CHAIRMAN NORQUIST: Go ahead.

DR. DOUMA: Allen Douma. Just on the bar graphs you were just looking at. Two things. One, the out years commitments. Can you talk about how we came up with those numbers? And also just looking at the bar graphs themselves, it looks like our revenue is greater than our commitments through the life of the organization. Can you talk about that?

DR. SELBY: Well, that shouldn't be. Those should add up, and I suspect that they do. And when you say "out years" you mean the remaining years up to 2019 or are you talking about --

DR. DOUMA: Yeah.

DR. SELBY: Sometimes we talk about the years beyond 2019 as the out years.

DR. DOUMA: No, `16, '17, '18, '19. Where do we come up with those commitment numbers?

DR. SELBY: Those commitment numbers have been with us since about 2013, was the first time that we -- Christine Goertz, go right ahead.

MS. GOERTZ: Thanks. Christine Goertz, chair of the SOC.

All of these numbers are numbers that we've been looking at our budget and projecting now as far as our research funding strategy goes, for the last at least couple of years, right to the end. And so, we -- remember the research funding strategy that we looked at last year in our retreat that had basically all of these numbers in it. So, when we're -- and not only do we have this -- when we're looking at our revenue and our commitments, we also have some targeted idea of how much we might want to spend on targeted funding announcements in our pragmatic trials.

And so, the main agenda item for the SOC tomorrow is to be re-looking at this -- at our research funding strategies from this perspective and seeing if we want to make any changes from what we've -- that we would want to recommend any changes to the Board based on the -- you know, the ever-growing knowledge that this is not -- we don't have infinite money and we want to make sure that we're making the very best choices possible.

DR. DOUMA: Joe, the reason I mentioned

about the revenue commitment mismatch -- if you look at the middle two columns where the commitments are somewhat higher than the revenue, almost everywhere else it's the same where the revenue is significantly higher than the commitment. So that's why it looks like it's not a zero sum game up there.

DR. SELBY: I agree with you. Not quite sure how that came to be, but you're absolutely right that the green bars, if you added them up, should equal the blue bars.

Well actually, no, that's not true. You're subtracting -- we're not showing here the non-commitment portions of our expenditures. So, things like keeping the lights on.

DR. DOUMA: Oh, okay. So this is just research --

DR. SELBY: Yes, this is just research dollar commitments. Right.

DR. DOUMA: Got you.

MR. BECKER: And I think, Joe, there are out years, right? So some of these dollars could

have been continued to get funded '20, '21, '22 --

DR. SELBY: There's no new commitments though Larry. That's right.

MR. BECKER: Right.

CHAIRMAN NORQUIST: [Off microphone.]

MS. GOERTZ: New commitments.

DR. SELBY: Both the revenue and the commitments end in 2019, at the moment.

DR. KRUMHOLZ: If I could just say very clearly for anybody that's listening in. And this is directly to Alan's question, I think. It is not our intent to commit less as of the end of 2019 -- to commit less than the full amount of our revenues. In other words, the idea is not to keep some reserve or something of that sort, except to the extent that we need to have dollars to pay for the tail on awards that are made prior to the end of 2019, as we continue to administer those out until the conclusion of those particular awards.

DR. SELBY: Good, so this is just background to keep in mind. I think it reflects a rather subtle change in our awareness of the entire

context in which we operate, and it will play out at the SOC, among other things as Christine mentioned. Do we have the right amounts in the broad, pragmatic clinical studies, and the targeted announcements?

So, completely different picture, completely different topic, equally big topic. This is about PCORnet. And there's a lot of things we can say about PCORnet, from how we engage patients and clinicians and other stakeholders to make it a truly patient-centered clinical network, to how we support individual projects in it and the investigators affiliated with the different networks.

But another part -- and I really want you all to be aware of this, and I know it was in your thinking when you first approved the funding for PCORnet -- is PCORnet would become, among other things, a part of a national clinical data research infrastructure that would mean that within the United States we can conduct more, greater numbers, larger patient-centered comparative effectiveness

questions. But while you're at it and you built a network, you can also conduct a lot of other types of research that has the stamp of patient and stakeholder engagement in governance, has the stamp of highly-standardized data, and has the stamp of being more efficient and more affordable than the models which we currently have, which really severely restrict the amount of research that can be done.

So, this is a picture that was presented, actually, not by PCORI but by Janet Woodcock from the FDA at a meeting that was called a "summit". And it convened the leaders of the FDA, including the deputy commissioner who is now nominated to become the commissioner, Rob Califf, but also the heads of both CDER, Drugs; CDRH, Devices and Diagnostics; and CBER, the Biologics. Those three centers, their leaders were also there.

From the NIH we had top leadership and persons of Mike Lauer, Josie Briggs, who is acting director of the Precision Medicine Initiative, and Petra Kaufmann, who is from NCATS and runs the

CTSAs. Critical fixtures also in any national research infrastructure.

And this was Janet's vision, that through -- by creating a common data model in various sectors of healthcare enterprise, including in providers the hospitals, physicians, and integrated delivery systems and bringing patients along -- and if you don't recognize it, that is PCORnet -- as well as in the upper part of the circle, the payers, the public and private payers. And if you don't recognize that, it's essentially Sentinel. So, Sentinel is the FDA's major contribution to a national clinical research enterprise, mostly focused on safety, but increasingly interested in being able to do comparative effectiveness as well.

Now, the beauty is that we chose the same common data model as Sentinel. So we are now working very hard with our announcement that invites health plans to join us in PCORI in collaborative research efforts using the common data model and identifying overlapping patient populations to further this notion of a high

quality research network operating at a national level of 10s if not 100s of millions of persons.

Critical realization is that you can't do good research without both electronic health record data and claims data. Most settings in the United States don't naturally have both of those. That's why the linkage between PCORnet, on the one hand, and Sentinel on the other is very crucial.

The third critical player in any national research enterprise would be registries. There are hundreds if not thousands of registries, many of them extraordinarily rich, detailed information on many people who either have a condition or who have had a procedure. This enterprise needs to be able to work with those registries.

Once you have that distributed national information network, any number of users can work with it. The FDA can continue doing its surveillance through their coordinating center. Industry can sponsor medical product safety research through the same coordinating center or another coordinating center. Any coordinating

center should be able to submit queries and get results, provided the network and other patients and governing units in the networks approve the research.

Clinical research, whether it's funded by NIH or it's funded by industry can do the same thing. Comparative effectiveness research founded by PCORI, certainly, but also by others, can use this national data network. But it's also useful for surveillance and it's also useful for monitoring quality of care.

So, this vision is a vision that's shared now by the FDA, by the NIH, by PCORI. I want to say that the Reagan-Udall Foundation was also in attendance at this meeting, as was CMS and ONC and the Office of the Assistant Secretary for Planning and Evaluation.

So, it's an idea whose time has come. It's on the minds of a lot of people and PCORI is the first national enterprise to attempt to harness the electronic health records portion of it and to bring the patients and other stakeholders in along

the lines that we've just discussed. So, that's the second idea, I just wanted to mention. We will be talking about PCORnet at every meeting we have from here on out, and this is a part of the picture.

Yes, Sharon.

DR. LEVINE: Just a clarifying questions. When you say good research is hard to do without both claims and EHR data, do you mean on the same patients? Or do you mean --

DR. SELBY: On the same patients. So in other words, the electronic health record data is far superior for characterizing who the patients actually are at the time they get a particular treatment, and often for giving you the details of the treatment.

Once they leave that hospital, once they leave that doctor's office, the electronic health record has no claim to what happens to them afterwards. They may go to places that don't have the same electronic health record. But their insurers, whether they're public or private, tend

to learn about everything that happens to them. So, the payer data, the plan and Medicare/Medicaid data, they tell us who is still under observation and what's happening to them inside or outside the system.

Most of our studies are longitudinal outcome studies, so you need both.

Other comments? Okay, I see two. Yes, Ellen and then Harlan.

MS. SIGAL: So obviously very supportive of this and I'm extremely supportive of the synergies because I think they're really important. It just seems that with Sentinal, PCORnet, you're all -- we're all using the same people for methodology that the connection would just be absolutely critical and synergistic.

I know that FDA for a long time has talked about that. Janet -- and I know Rob has -- we're all on the same page. I guess what I don't understand are what the next steps are to see how these networks can combine and really have a critical mass and really have some outcomes. So,

how does this happen? What are -- other than saying there's a lot of synergy and we should work together, what should we do? What are specific steps?

DR. SELBY: Well, I think PCORI and PCORnet have a lot to do in bringing these health plans on-board and beginning to work out some of the tricky issues about how you actually do collaborative research when a series of providers, hospitals, delivery systems, and payers have to share data in order to conduct the research. So, that's a series of discussions and policy-building that has to take place.

The secret will be in conducting research that matters to both parties. But I think in the background, PCORI, DFDH, and NIH can create a favorable environment for that to happen. Can make it clear that this is a vision and in fact, one of the conclusions of that meeting in late October was that we would write a manifesto, which basically said on behalf of the player organizations there is a lot of energy. There is a conviction that this

is the way research needs to go.

Harlan.

DR. WEISMAN: Joe, in addition to evidence generation, which is shown here in terms of the power of the network, I wondered in the spirit of a continuous learning healthcare national system. We are evidence synthesis and evidence query -- how that fits into this, and could it fit into this? In terms of vision.

DR. SELBY: That's a good question. And I think, you know, if there's more of a linkage between those who generate the evidence, the notions of standardization, for example, of data -- which underlay a capacity to actually do evidence synthesis -- could be furthered. I will say, I don't think there was a lot of talk about evidence synthesis at the summit, but it's a very good point that by having more unity at the time that questions are posed and data are collected, evidence synthesis should be furthered.

CHAIRMAN NORQUIST: Christine and then we'll go down this way and come back over to

Barbara.

MS. GOERTZ: Thanks. Joe, I was wondering at this meeting if there was any talk about the long-term funding future? I know that the NIH collaboratory Common Fund, funding is going to run-out at approximately the same time as PCORI or funding for PCORnet will also be termed out, at least for this initial phase at 2019.

I'm just wondering if there's any discussion about that and what impact that might have or what people are doing to think about additional funding sources?

DR. SELBY: Mike, would you care to comment on that from the collaborator point of view?

DR. LAUER: I think part of the thinking here is that -- I think this is a lot of what was said during that seminar on October the 28th. Is that if this model works, the model is already happening, to a large extent. Many of the trials that FDA is overseeing is based on this common data model.

So, if this model really works, then it would be possible to, in Rob's words, enroll 10 times as many patients at one-tenth the cost, even if you can only do a small fraction of that. And that will then enable this business to move forward.

I think the idea here is to develop a new business model for conducting clinical research. Not just in our sphere, not just in the NIH sphere, but also through the entire sphere, so that it's possible to do a lot more research for a lot less money, and a lot faster.

MS. GOERTZ: I have one follow-up question. What does that mean as far as barriers to access for investigators? If this is going to be the new model, how easy will it be for investigators to have access to this data or to be able to --

DR. LAUER: -- knock down the barriers and make things a lot easier to do this right.

DR. SELBY: I think that, you know, we are to some extent swimming upstream on that issue.

But the Board's support of this along with support from other agencies will be important and I think we'll prevail.

CHAIRMAN NORQUIST: So, Harlan, Alan, and then Barb, and we'll stop this so we can move on to the next topic. Okay, Harlan?

DR. KRUMHOLZ: I have just one comment and then a question. You know, I think this is really important to align it. I just want to make a comment that I think distributed data models have yet to show their worth. The Sentinel initiative, by the way, has received a lot of funding and has yet to really demonstrate what its home runs are. The difference between being able to collate data like you can in the VA and being able to look at it at a distance is an experiment and an experiment which I think should show whether that's going to work or not and produce the kind of knowledge that we hope that it would.

Just a minor comment again is that this is something we need to study carefully because if that's not going to work we need to know as soon as

we can and it needs to be tested in various different ways. You're being smart about aligning with this group, I just want to be sure we're -- you know, we should be providing some of the funds to study that as well.

One question I had about this is, so we have all of this data now. What is our position on the degree to which patients know that their data is being used in research from all of these places? And as it gets used in various different ways, what are the assumptions about their knowledge and involvement and engagement? Is it being done in front of them or behind their backs?

DR. SELBY: Good question. You know, I think that our current position is that a lot more dialogue has to be had with patients about practices that have been going on for 40 years. I mean, this is nothing new here -- and about the value of the research that comes from that -- the amazing value of some of that research. This trustworthiness meeting that Sharon put on the table is one of the forum in which that will be

discussed. We really need to have ongoing dialogue with patients about that.

DR. KRUMHOLZ: And I just want -- so a tweet that I did yesterday. There was a newspaper article that said that Watson Health was touting that they had 100 million electronic health records. And so I just tweeted that's great, I wonder how many of those 100 million know you have their health records.

You know, I think we can be in sort of an important position of saying that there are ways to do this that people can know about it and agree to that can still be very strong. I know you're fully on-board about this so it isn't a criticism; it's just a comment about the kind of role that PCORI can play in helping to bring this dialogue forward.

CHAIRMAN NORQUIST: Excellent point, Allen.

DR. DOUMA: You say in your report that we read -- previous material that says only by bringing these two resources for patient capture into both networks will we be able to maximize the

value of either. Question is, can you characterize how bad it is if we only have EHR so we're not maximizing -- I'm not sure what that means.

And secondly, a follow-up to Ellen's. Do we have a timeframe in which we think it will happen? I know we're working on it hard, but things like this take a long time normally. How long do you think it will take for us?

DR. SELBY: We'd be pretty limited in the kinds of CER studies we could fund if we don't have claims data.

Now, let me say that many of these networks have been trying to capture claims data on their own, and some of them have succeeded nicely. So parts of our network -- and then parts of it had claims data. For example, the integrated delivery system had claims data from day one. So, in some quarters of PCORnet you can do good longitudinal outcomes research right now. But in others I think, you know, it is going to take a linkage to Sentinel systems to get claims data on the majority of patients and that's going to take probably two

or three years and it will move ahead from project to project. So we will not -- nobody's going to do it en masse. It will be IRB-approved project after IRB-approved project, and building the case and learning as we go.

CHAIRMAN NORQUIST: Barbara.

DR. McNEIL: I think this is a great model. I have three comments. The first one relates to something Harlan just said, and that is the Sentinel network has received a lot of money. And from a personal perspective, I would really like to know what its successes have been because I actually don't think there have been too many. And if there haven't been a lot, I'd like to know why. And I'd also like to know -- because it will affect this whole model -- what the barriers to entry are or are not for that particular activity in your upper left-hand corner.

So that would be my first comment. The second one is in terms of the FDA's involvement, I think we're going to be a little bit stuck on this model for Devices until a unique product

identifier, because all devices aren't the same and a hip is a hip even though it might be different from three or four different manufacturers. So we just have to put that in mind when we think about this.

And the third comment is when we think about this model it's my understanding that the two largest groups that supply electronic medical records are Epic and Cerner and I suspect they have -- I don't even know, somebody here would know what percent of the market they have. But it's huge. Sixty or 70, Harlan? And it's also my impression that Epic isn't willing to at this moment aggregate up all of their electronic health records from say their 50 percent or 40 percent of their enrollees up to some central database.

So if they're not willing to do this, it's not exactly clear to me how Part News is going to pal up with Yale, for example, in terms of sharing data. I just don't know how we would do that. So I think some of these details will be very important work out as we move forward on this

model.

DR. SELBY: Let me just say, you're right and those are all important issues, but I don't think we have time to -- that's an hours' worth of conversation, interesting conversation.

CHAIRMAN NORQUIST: She's just making a comment about the importance. And then, Ellen has the last word here and then we'll move on.

MS. SIGAL: That would be so nice to have lost word. No, specifically I think there is a lot of synergy that's tangible. I think, Barbara your question about devices or statement about devices is correct. I do think with Sentinel and PCORnet and Reagan-Udall with IMEDS, that is an accessible network that can be linked and there are very tangible outcomes that can come out of it, but this is a longer conversation.

DR. SELBY: Two really good points about Sentinel. Number one, it wasn't set up just to do published research. It's much more of a surveillance tool for the FDA in much of their work goes on behind the scenes. I think if you talk to

the FDA they're quite pleased with the way Sentinel has been able to really rapidly turn around analyses that help them know whether to be worried or not, so that's one point. And the second is, the point I made before that without EHR data Sentinel cannot create some of the really rigorous studies that could show up.

Okay, so I just want to briefly mention four meetings just so that you know that we are continuing to engage at a frequent and high level. There is a meeting coming up in December, and also to invite Board members to tune in to most of these meetings by webinar or to come in person. A very interesting meeting December 7th. Pardon? That's not right is it? December 17th, I think it must mean. I apologize.

CHAIRMAN NORQUIST: Tenth.

DR. SELBY: Tenth. Good we have Robin here. Thank you. So, this is a topic that is really highly relevant to PCORnet and to the PCORnet Sentinel collaborations if and when they happen. It's really originated from the

Methodology Committee, if I understand it right Robin.

It is not an open meeting, but the transcript of the meeting will be posted on the PCORI website very shortly after the meeting happens. The second is an expert stakeholder working group considering the question of back pain, and particularly the question of comparative effectiveness of surgical versus nonsurgical approaches to chronic back pain. In this is actually the third expert work group we've had on back pain in we will have a range of stakeholders to consider the very interesting question of what subgroups of back pain patients is this really most relevant to? In other words, there's some patients who there would be probably little argument would benefit from back pain surgery. Some in whom it's kind of clear that they just shouldn't be taken to surgery but for the patients that we invite, what subgroups should they come from and what subgroups should we be careful to measure?

So it's all part of refining the back pain

questions before we bring them to the Board for review and approval in January. The meeting will take place in January, you will hear from us after this meeting. And that is a meeting that is being conducted with webinar capability for participation and details will or will be shortly on the PCORI website.

Next is the trustworthiness meeting, I won't go into further detail because Sharon already took you through it. That's January 19th and 20th. It's all about engagement. Similarly, all about engagement is a meeting on the 20th and 21st at the National Academy of Medicine. This is a follow-up to our 2014 meetings and we will be sitting down with health system and health plan CEOs to look at and prioritize a set of questions that PCORnet could help these system leaders address within and across delivery systems. So another open meeting that I would certainly invite your attendance or tune-in online.

CHAIRMAN NORQUIST: Harlan Krumholz.

DR. KRUMHOLZ: I was just going to ask

Joe, these are terrific and in particular the data quality. Is there any chance, you said it's not an open meeting bot were going to get the transcript, why not stream it? You could make it so people can't ask questions and so forth, but I just think these are so important and will be so great to do that.

CHAIRMAN NORQUIST: Robin.

MS. NEWHOUSE: So the intent of this meeting actually came from the Methods Committee, a PCORnet subgroup around specific data issues. The data quality was one; Sally Morton led a group of PCORnet and other investigators to talk about those issues. So, it was intended to be more of a Methods discussion and try to understand the gaps are PCORI Methods could fill, so wasn't thought of but the question about why we can certainly take that back to the group.

DR. KRUMHOLZ: I'm certainly thinking that I would recommend my postdocs listen in. I mean, I just think there's probably a lot of wisdom and even if it's the dirty work of making sausage

that's the part that would be wonderful to show people how they are struggling with it.

DR. SELBY: We will definitely take this under advisement. I'm just going to -- in the interest of time move on and I don't know if I push a button now or is Bill does, but this is really -- very much follows on the discussion that we have a Sharon and Sue, this is a product, a video made by researchers at the Center for Health Research in Portland about a large PCORI-funded project that they have had underway.

Here we go.

[Video shown.]

DR. SELBY: We showed this for two reasons. One, because it suggests again, reinforces the notion that research and clinical care will really change if we engage patients in both of those crucial activities.

The second is because it's a nice example of the importance to patients of the research that we approved at our last Board meeting on the treatments for patients who are currently with

chronic pain and on high doses of opioids, so for both of those reasons.

I will move ahead and just mention briefly what else is in store for the Board meeting today. We will have an end of the year Dashboard review just after this, and then a presentation on new data, new data elements, that will allow us to ask questions about our portfolio that we have not been able to ask to this time, so this is in response to a Board request. Lori Frank will be making that presentation.

Right after lunch, we will hear from a panel of patient stakeholders, including Marc Boutin from the National Health Council, Donna Cryer from the Global Liver Institute, and Celeste Castillo Lee from the Institute for Patient and Family-Centered Care, in our continuing series of engagement and dialogues with key stakeholder groups.

Then we will consider two new standards. This is standards for issuing public comment, and the second is revised Selection Committee Charter.

After the break, we will hear -- actually, I think at 2:15 is actually the Methodology Committee report. Sorry. A report of some analyses that Board members and staff members have been actively involved in on how our merit review scoring works, and then a proposal on what we have never really discussed, workforce training, but we have been -- it's mentioned in our legislation, much of the responsibility lies with AHRQ.

We have been talking with AHRQ. We have been talking with the Research Transformation Committee about possible activities in the area of workforce training.

The last item on the agenda will be open science, a presentation on progress we have made in moving from policy to procedure in open science, and wrap up with public comments.

I realize that we have gotten behind, even though we started ahead with those two pictures. They invited more discussion than we should have.

DR. SELBY: I'm going to try to go pretty fast through this Dashboard, although I will say to

you in advance, I am looking for new elements on the Dashboard. I love our Dashboard, but I'm getting a little tired of some of these boxes. Suggestions for making the 2016 Dashboard newer and even more compelling will be welcomed.

I am also going to show you some evidence again on the funding commitments, which will lead into a discussion about our approach to funding.

The same three yellow boxes as you have seen before suggests that we underspent in 2015 compared to how much we had planned to commit in research funding. I want to say one thing about that, how much we planned or how much we budgeted for.

We always took the upper limit of when we said up to X millions of dollars, we always took the upper limits of that and added. Arguably, we shouldn't ever come quite to what we budgeted in the year. Nonetheless, there was somewhat of a shortfall, although you see we made up some dramatic distance in the fourth quarter of 2015.

Projects completed. Those are mostly the

pilot studies at this time, and basically after some adjustments of milestones and target dates in about half of them, those projects are all complete now, and we will have more information on that.

The bottom one again shows we also underspent in staffing and other non-research categories in 2015, and we have discussed that before.

I think elsewhere on here, I'll point you to the very middle box, which shows that the number of articles published by our awardees, PCORI funded researchers, has gone up quarter on quarter, and now they are appearing at the rate of well over 100 per year, and I'm sure that will just continue to rise as more and more projects come to conclusion. Also, the number of papers written about or by PCORI has increased a bit in the fourth quarter.

Over in the upper right-hand corner of the milestones, we will drill down on that because you had a lot of questions last time about what happens, how do we monitor projects, and what is this milestone adjustment and other adjustments we

made.

The web views are important because this is on the uptake of methodology standards. These are web views to our website of the methodology standards. You will see they remain very high and even were higher in the fourth quarter, and this probably to a substantial degree represents applicants who were checking on our methodology standards.

The box to the right, the citations, is rather high as well. These are citations to a particular article, and I'll show you the article in a minute, written by the Methodology Committee at the end of 2012 as the standards first came out. That was in JAMA. That article continues to be cited.

This is a nice example of a project, a course. This is a health psychology course for a Master's program, and it zeroes in directly -- there are 10 modules and six of them are focused on PCORI. They focus particularly on PCORI's research priorities. This is at Tulane.

It is nice because as you will see, we fund an amazing amount of research in behavioral health. This just shows year by year, these are annual commitments. We have moved dramatically from 2013 where about 80 percent of the funding was delivered in the form of broad, awards for broad announcements, to 2015, where well over 75 percent -- I'm sorry -- about 70 percent of our funding was either in the targeted announcements or the pragmatic clinical studies, these larger more focused studies.

Cumulatively, you see it begins to have a larger effect on our overall portfolio. Now, our portfolio consists of about 60 percent of broad's and about 40 percent targeted, but as you can see from the earlier annual trend, that will change, and the increasing proportion from here on out will be the larger more focused studies on big questions.

Just to remind you about the topic prioritization pathway that the SOC approved last year, the topics continue to move through that, and

they wind up either on the targeted PFA list or the pragmatic studies list as high priority questions.

This next slide, I like this slide a lot. It shows the progress of questions that are in the sort of intermediate stages, lists four and five on that pathway, to those topics that have been approved either for placement on the PCS announcement or for targeted PFAs in the middle column to those that have been funded, those topics funded through the targeted PFAs or topics funded from and approved through the pragmatic clinical studies initiative.

A lot of ideas moving through PCORI. We feel the pressure to move these through in an important way to be open to staff's late breaking questions that we have to be amenable to as well. This is the work of the SOC. They have a lot of work, and the work of staff.

This is the distribution of projects by topic. I think if back in 2011 we had decided to prioritize the diseases that we wanted to study, it would look probably a lot like what we wound up

with through a much more open process. Quite interesting.

The one thing that surprises me personally just a little bit is how mental and behavioral health has forced its way to the top of the distribution. I think it is really telling. I think it says that --

CHAIRMAN NORQUIST: Particularly in the last --

DR. SELBY: Where the problems are. It has nothing to do at all with the composition of our Board of Governors nor with its current chair person.

[Laughter.]

CHAIRMAN NORQUIST: By numbers, what is the --

DR. SELBY: Same thing, basically the same thing. The majority of our studies in our portfolio are clinical trials. I remember when we used to talk about how this would look, and it looks like in our peer reviewers and our staff and SOC, Selection Committee's judgment, that it often

takes a clinical trial to do good comparative effectiveness research.

This is a focus on the care continuum from prevention to screening, diagnosis, to treatment. We are still very heavy whether you look at it by number of projects or by the amount of funding, still very heavy on the treatment side. Most people are probably not too surprised by that, but I think Francis pointed out this one time, that we should probably be looking for more ways to fund more in prevention, screening and diagnosis.

This is just the way it has gone in terms of our programs. By design, more of it has gone to the assessment of prevention, diagnosis, and treatment options, CER, at the individual level, but improving health systems and addressing disparities actually have sizeable pieces of the total portfolio, reflecting their importance. Methods and communication dissemination research, again, they are small as much by design as anything else.

I think this is a pie chart that the SOC

will continue to look at and decide whether this makes the most sense.

You have seen this before. This is just simply the way we monitor the progress of projects. You will hopefully get more and more familiar. I'm still familiarizing myself with it, if that makes you feel any better. Green, yellow. Green is good. Red is really bad. Yellow and orange are in between.

The next slide just shows if you start at the bottom, that is quarter two, then going up quarter three, quarter four, you will find that the number of projects have increased, that the distributions are not too much different, maybe a little bit of a larger fraction in the green category, but a certain number in the yellow, orange and red as well.

The black box is number one. You had asked before about whether any projects have been terminated. One project each was terminated in the third quarter and fourth quarter. The reason we didn't tell you about the one in the third quarter,

at the end of the third quarter, is because we were still defining exactly what it meant to be terminated. Now that we have the definition, which is the notice of termination has been issued, we have one in each quarter.

We had a lot of questions last time about contract modifications. This shows the reasons for the modifications. We can modify a contract to remove or add milestones, and these are the percent of projects in each quarter that have had milestones modified.

The scope of work can be changed. It may just have been they were too ambitious and we ultimately agree mutually that the scope of work ought to be narrowed.

The period of performance has been lengthened, and I think the reason these are as small as they are is because it is still early in the life of many of these projects. I think the numbers will go much higher in terms of the number of projects in which we ultimately agree with the investigators that the period of performance should

increase.

Then a very small fraction, one quarter, where we agreed with the investigators that we actually needed a budget increase.

This is the pilot projects, just the pilot projects. The pie chart on the left shows almost half of them requested and obtained a contract extension. That is why you see most of those two year projects ending in 2015 instead of 2014.

This is probably not too unrealistic for what we are going to see long term with the larger projects, although because the pilots were limited to three years, there might be a little bit more reason for them to look for an extension.

The pie chart on the right just shows that given the extension, 88 percent of projects came in on time with the modified time line, still 12 percent that had not completed at the expected end of the project.

This just shows you that projects will be terminating, this is a question that Allen Douma asks, as do others. You will see that 2016 is a

year in which we expect a number of projects, a number of our comparative effectiveness research projects, not the pilots, so the green bars, to terminate. 2017, a huge bolus, that will probably spread out a little bit in future iterations, and some of them might get modified to be terminated in 2018 or 2019. Large number of --

DR. WEISMAN: Completed or terminated?

DR. SELBY: I'm sorry, thank you. Completed. Absolutely not -- I'm sorry to everybody including those listening at home.

DR. WEISMAN: Can I ask a clarification on completed? I guess it means -- are we meeting our statutory requirement of reporting results within the time frame of completion?

DR. SELBY: I think that process starts just at the time they are completed. That's another very important topic, we are setting up the infrastructure to do that right now, so yes, I don't think there is any concern here that we won't meet that requirement.

DR. DOUMA: Joe, quick follow up to that.

How much does it change that pie chart to see basically the first date on which we can see the results, and the follow on to that is can we have a list of those things specifically project by project?

DR. SELBY: I think that's a good idea. We can work to provide the Board with more information, maybe even be somewhat more informative on our website about when projects are expected to come to completion.

This is just the number of journal articles. This might be affected a little bit by the fact that we now have a full-time librarian who is very good, and she may be delving into and identifying those articles a bit faster.

I think it also is a real trend of increasing studies. This is year on year. The 110 is -- the top blue line is articles in 2015, but 2015 showed increases in every single category, articles by or about PCORI and articles that cite our mission and PCORI work, as well as funded research.

Among those funded studies, there really are a lot of papers published on the protocols for CER trials. This is just three of many where the investigators have taken the time to publish their protocols.

This is another more detailed look at the up tick of our methodology standards. These are data provided from JAMA, in fact. These are the number of citations of the methodology standards, the citations of this single paper on the methodology standards in JAMA in 2012. There is the reference at the top.

The number of citations, but also the number of views. That is a lot of views. The number of downloads, over 1,000 downloads of this article. People are looking for information on the methodology standards and finding it.

This is another slide relevant to the dissemination of the methodology standards. This is a course that PCORI funded the development of with Baylor University, to develop an online course for clinicians of all types to get familiar with

the methodology standards.

There are six modules. This has been up. In future Board meetings, I will be presenting to you tracking data on the use, the number of people who have been certified with this CME. It's directed to all sorts of clinical groups, not just physicians, and Baylor is doing a lot of work to publish and disseminate the availability of this training module.

UNIDENTIFIED: Joe, just for people listening at home, if someone is interested in doing this course and CME, where should they go?

DR. SELBY: It's on PCORI's website; is that right?

UNIDENTIFIED: It's on the home page.

DR. SELBY: Thank you. This is one other course. Another interesting course developed at the University of Pennsylvania totally focused on the methodology standards. This is a course in patient-centered outcomes research. The actual course is called Methods in PCOR and Effectiveness Research. Quite nice they have a class dedicated

to each one of the standards in the methodology report.

Now let's have a brief discussion, but we have talked so much that I really want to wrap this up now within a couple of minutes and hand it over to Lori Frank.

Gail?

MS. HUNT: With regard to that last example that you had up, Joe, there was an earlier one from Tulane where they had developed a class as well or a course. I guess I'm sort of hoping that PCORI is not funding a bunch of universities to do individual courses that then --

DR. SELBY: No, we did not fund either of those. We funded the CME for the methodology standards, but we did not fund the development of the course at Penn or Tulane.

MS. HUNT: Okay; good.

DR. SELBY: Those are courses that are springing up so that people can understand PCOR and PCORI.

MS. HUNT: Yes, but I still think it would

be great if we sort of intervened or if we at least gave them the opportunity to say, you know, we will be happy to help spread this across the country to other universities so they can benefit, even though we didn't fund it.

DR. SELBY: Good; thanks. Sharon?

DR. LEVINE: The comment was about the three slides beginning with the graphic showing the process of approval followed by mapping topics to that. Those two slides and the one that follows are the three most effective communication tools I've seen yet to try to explain to people what PCORI is all about. They're fabulous. I wish we had them a year ago.

I think it's an extremely effective way to respond to queries about what is this all about.

DR. SELBY: I'll just give a nod to our team, Michele and her team, for visualizing them in ways that are useful, and to actually Science and SOC for the one you like, the pathway.

Barbara?

DR. McNEIL: Two comments, Joe. Do you

have any idea how often grants that fail from a methodologic point of view have had PIs who have reviewed or looked at the methods website?

DR. SELBY: No.

DR. McNEIL: Maybe it would be a good thing to find out. The second point to that is would you ever think now that we have many letters of intent and we have some sense of what individuals are doing when they submit a letter of intent and we say yes, go with it, you say yes, you're going to do X, Y and Z, think about looking at method sections A, B, and C, or look at module six from Baylor, or whatever, just to potentially increase our acceptance rate and cut down the number of poorly executed grants from a methodologic point of view.

DR. SELBY: Nice. I will say, Barbara, that if anybody applies, even to write a letter of intent, online, the online page that they are working on has a direct link to the methodology standards, and they are admonished to check them.

I don't think we have gotten as

sophisticated as saying oh, you're doing a cluster randomized trial, we will have a cluster standard next month that you can look at. It's a nice idea.

DR. McNEIL: I was just being a little pushier.

DR. SELBY: Yes, more targeted. Debra?

MS. BARKSDALE: I have two quick questions. On the slide that you talked about the reasons for contract modifications, are the reasons mutually exclusive or are some studies --

DR. SELBY: I don't think they are mutually exclusive; no. If anybody here knows they are, correct me. I think a contract could be modified for two or three of those things at the same time. Milestones and scope, for example.

MS. BARKSDALE: You don't know right offhand how many studies this actually represents?

DR. SELBY: No, I don't. Let me turn quickly to Michele.

MS. ORZA: No. [Off microphone.]

DR. SELBY: We did not ask ourselves how many studies had some kind of modification. It's a

good question. We will get that back to you quickly.

MS. BARKSDALE: My other question relates to the number of publications. You answered it in part. You said there are a number of studies on protocols. What are the kind of things that people are -- not the papers that are written about PCORI but the ones from funders, people who have been funded, what kinds of things are they writing about? It's not research findings necessarily; correct?

DR. SELBY: There definitely are a number of papers now about research findings. I presented three of them last time, for example. There are more of those. There are some evidence syntheses that were done at the beginning of a project. There are some descriptions of the tools that were developed. There are papers about what it was like to engage with patients.

In addition to the protocols, those are some of the themes that come to mind.

MS. BARKSDALE: Thank you.

DR. SELBY: Thank you all very much, and now thanks to Dr. Lori Frank, I remember a little over a year ago, Harlan Krumholz really urged us to get more information on our portfolio and be able to look at it in a variety of different ways. I actually charged Lori with that, and she has just done amazing work.

This is really a way to bring you up to date with what is available at PCORI now. Knowing what is available starts to inform the kinds of questions you want to ask.

Lori, thanks very much.

MS. FRANK: Thanks very much, Joe. I do want to acknowledge this is a team effort and a large team at that. Some of the team members are listed here. My thanks to Rachel Witsaman, who has been an internal leader for this. Vadim Gershteyn as well, who is back there. We are so glad to have Heather Edwards who joined our team. Heather is back there.

We worked with a team from Ohio State University as well, and I'll introduce them, but

they were led by Dr. Ann Scheck McAlearney and Dr. Timothy Huerta. They brought us some additional clinical expertise, health services expertise, coding expertise, and medical librarian expertise.

What I would like to do is bring you up to date on the PCORI portfolio taxonomy, describe how it was developed, and introduce you to the structure of it. Then we can walk through together a few examples, which I think help bring to life the structure, and my goal is to have us all think jointly about how best PCORI can leverage this as a tool.

The goals for developing this were twofold. First was to support reporting. As you saw, Joe just went through, we always report out to the Board and to the public on the conditions that PCORI is funding studies in, what the study populations are. We give information a bit about the comparators, and also we describe where on the care continuum these projects are located from prevention all the way through treatment and transition.

We wanted to enhance our ability to report on the contents of the portfolio to better communicate to everyone about what PCORI is funding.

The second goal is to use that enhanced information to support strategic decision-making. With a greater level of detail, we feel like we can better understand the direction PCORI should go in in the future. This will also enable us to be able to take a close look at what research teams are bringing to PCORI in terms of their ideas and what stakeholders have been bringing to PCORI in terms of potential topics and compared against what we are actually funding.

Wherever possible, we built from precedent, so you can see we turned to the unified medical language system, UMLS, MeSH headings, Census definitions, and we also referenced the NIH RCDC, Research Condition and Disease Categorization, in this process.

The structure of the taxonomy is hierarchical, so I'll show you that we have high

level themes. Here we are using an example of population. Beneath the themes are codes. The example here are age groups within the study population, and below that are sub-codes, and we have sub-sub-codes, which you will see in a moment.

I mentioned that we built from precedent in terms of content, but certainly in terms of structure as well.

The reason why we couldn't just use one of these great systems that's already out there is because the PCORI portfolio is so unique. There are some ways in which we wanted to express that uniqueness.

First, about the populations that are being studied, next about the nature of our comparators, third, you will see greater attention to the outcomes within PCORI funded projects. We can really now describe those projects in great detail. Finally, a huge unique aspect of PCORI's funding is being able to capture and describe the stakeholder engagement in the research that PCORI funds.

This is the most fun I've had in a very long time, showing you this.

[Laughter.]

MS. FRANK: These are the four categories in red that we have been reporting on, conditions, study population, care continuum from prevention through screening, diagnosis, et cetera, and comparators.

This is what we now have. That was so much fun for me I'm going to do it again. So, this represents a great deal of work and thought and input. It is still a work in process, so we are interested in your input as we move along.

I just wanted to share with you that we have started at the level of a study, that's the blue box at the top, and we now have an additional seven themes for 11 themes total, with a greater number of codes below each of those themes.

You can see the detail on the right there for outcomes. I'll walk you through that in a way that you can read in just a few moments.

Here's an example of the drill down.

Beginning with study approach, we have these five codes. What is the study method specifically, what is the study design, what is the randomization method, what is the method for data collection, and then what is the analytic method.

Here's the drill down on design. We have observational studies and interventional studies as the sub-code there. Then we have really excellent detail below that.

I'd like to turn to some very quick examples which I think will help demonstrate the structure of this and help us all think as I said through ways in which we can all best leverage this tool.

The codes that I'm about to show you are from a set of 252 coded projects across four of our five priority areas, assessment of prevention, diagnosis and treatment options, improving health care systems, addressing disparities, and communication and dissemination research.

We don't have methods in here because the methods coding is unique. There is some real

important differences, so we have a separate coding system for that part of the portfolio.

I'm showing you the coding results through everything funded through spring of this year. A really important point is that unless I say so, the codes are not mutually exclusive. They are inclusive coding. Every time a project had any element that met criteria for one of our code definitions, it got the check mark and we coded it as such.

The team began with the work that our strategic portfolio analysis team at PCORI began. The Ohio State group then had a great deal of consensus coding around themes and double coding, and once the code book was set, then we all coded.

Beginning with study populations. We have highlighted age, disability, and provider populations as examples we can talk through very quickly.

We asked the question which in this coded set of the portfolio relates to a focus on older adult populations, and 69 projects do. How are

those arrayed across the four priority areas here, and you can see that in the bar chart there. Then we can ask additional questions. What are the settings for those studies.

Of those that are noted to have occurred in a health care setting, what kind of settings. Forty-one of them are in ambulatory care clinics, two are in long term care facilities, et cetera.

We also have codes for the other settings in which these studies take place, a fair number of phone intervention studies, for example.

We asked the question which of the studies in this coded portfolio focus on vision and/or hearing loss, and the answer is eight projects, all but one of them focus on both. We can ask additional follow up questions, what is the nature of the intervention strategy for those projects. For five of them, it's provider or organization led intervention. Three of them have access to care interventions. Three have training interventions, et cetera.

Still within study populations, we asked

the question about providers, how many of these projects focus on community health workers, 15 do. We asked that same question, what is the setting, health care facility or other setting, so you can see quite a few take place in a home setting.

Another question we can ask is where in the health care continuum do these projects take place, so there are two on the prevention side, one on the screening side, and 11 in the treatment area.

Looking at study design, how many projects used a cluster randomized design, and the answer is 25 out of this initially coded set, and here is how they array across the four priority areas. What are the therapeutic areas or clinical conditions within those, and we can get the answer to that.

Still within design, looking just at that set that are observational studies, that is 24 percent, about 60 percent are prospective cohort studies and about a third are retrospective cohort studies.

We have a whole set of codes for

intervention. One of the codes is about the nature of the intervention strategy, and we can then ask questions about within access to care strategies how many used telemedicine, for example.

With comparators, our two main categories are two arm comparators versus greater than two arm comparators, and we have an overlay there with usual care information. Just as an example of a two arm comparator study, it is Anti-TNF alone or in combination with low dose methotrexate for Crohn's.

The three-arm study is a biologic alone or in combination with a disease-modifying agent, and the third arm relates to when in treatment it is introduced. A four-arm study where some interventions for improving adherence to cervical cancer screening guidelines being compared against each other.

I mentioned the greater level of detail for outcomes, so of course, we want to know who the reporter is for our outcomes in our portfolio, how many of these studies have a patient reported

outcome. How many turn to the caregiver as a source of info. How many are relying on biologic tests, for example.

That level relates to the focus for the outcomes. There is patient focused outcomes specifically or some that are focused on the level of the health system, for example. The Ohio State team really spent a lot of time on the nature of the concept in the outcomes. Health status outcomes, skills acquisition outcomes, health behavior outcomes, et cetera.

Finally, looking at stakeholder engagement in the portfolio. We code for the approaches. This is data you have seen before but now we do it based on the research plan. Are the research partners part of the team, are they co-PIs, are they on advisory boards, et cetera. Where in the engagement continuum are they being engaged, is it for study start up, is it for implementation, is it for dissemination or two or three of those. Who are the stakeholders who are being engaged.

For the set, we see that 91 percent of

these projects engage patients or consumers in some way, and almost the same number engage clinicians. About a third engage health systems.

These are just examples of ways in which we can now get our hands around some of the information.

Before I open it up for discussion, I want to acknowledge the work of our colleague, Elizabeth Harrison, a PCORI staff member, who led this work when she joined PCORI. She passed away about a year ago in October. We are indebted to her. We are grateful for the time we had with Elizabeth. We have the Elizabeth Harrison Fellowship now. It is one way to honor her memory and the work she has done for PCORI.

Team efforts. Here are some of the team members from the PCORI side and from the OSU side. I mentioned Dr. McAlearney and Dr. Huerta. That is their full team.

With that, I am happy to open it up to questions and discussion.

CHAIRMAN NORQUIST: Okay. We will start

from this direction. Mike?

DR. LAUER: Thanks. Mike Lauer from NIH. Lori, that was great. Are the individual projects coded manually? Is that how that is done?

MS. FRANK: Yes. We have been coding with the assistance of software, but it's one by one, so we can capture all the detail.

CHAIRMAN NORQUIST: Harlan?

DR. KRUMHOLZ: I just wanted to commend Lori for a terrific job. When I suggested this, it was not going to be easy, and it's easy to make suggestions, it's hard to deliver in such a nice and comprehensive way. I just want to publicly say thank you to the whole staff and to Lori for leading this.

As I look at this taxonomy, I think it would be an enormous help to a variety of organizations. The degree to which that documentation and the reproducibility of that method can be applied broadly, if it becomes a standard or it can be iteratively improved, we hold it as a living document or someone else does, I

think that would be very important.

Clearly, your guidance and your team's involvement have created something that will be very useful. I said to you I think the next step is to take the actual trials themselves and some of these areas could also be -- the same kind of thing could be done in subgroups.

I think there is still work to be done to continue to extend this, but search capability, the way we tag these, the way we understand the portfolio, the way we understand where we are getting progress and where we are not, all these things become a lot easier when we have a common language and a common way to look this up. It is a data standards issue, again, around the meta work that we are doing, not just the funding.

Thank you very much.

MS. FRANK: Thank you.

DR. DOUMA: I also want to say thank you very much. It is incredible to have access to the information and particularly historically to see what we have done, and at some point when we put in

completion of these things so we can grasp what is going on more on an ongoing basis, but from a more futuristic point of view, I'm hoping that the Selection Committee will figure out a way of using this in order to make selections.

Can you quickly tell them how they can use it and are they beginning to do so?

MS. FRANK: I'll let the Selection Committee speak for themselves. We certainly have discussed internally that this provides some context when the selection slate is brought before them. Absolutely, there are different ways in which they can see now how gaps are being filled on real time.

CHAIRMAN NORQUIST: Rick and then Barbara.

DR. DOUMA: Let me just quickly follow up on that. In order to fill in the gaps, we need to know where the gaps are, and the gaps are not simply what we have done, it is what we want to do. We have to have a picture, a vision, of what we want to get done before we know where the gaps actually are.

MR. KRONICK: I add my thanks and congratulations. It seems like great work. From AHRQ's standpoint, we would be very interested in kind of seeing the guts underneath it.

As a Board member, you may have said this, and I'm sorry if I missed it, is this tool available to us, and if so, how do we get to it?

MS. FRANK: Access it. Right now, it's available for staff. We're working on an interface that would make it useable for you and the public. We will report back when that is ready.

CHAIRMAN NORQUIST: Barbara?

DR. McNEIL: I agree with what everybody has said. I have one question, Lori. You gave a lot of statistics and percentages and whatever, and you had one I would have loved to have seen. One of these slides had the number of comparative effectiveness trials, A versus B or A versus B+ or A versus something with usual care.

Can you tell us what those numbers are from your data?

MS. FRANK: Yes; sure. We have about 70

percent in the portfolio that are two arm and the remainder are greater than two arm trials. There is about 55 percent for which usual care is stated as one of the comparators and it is specified. There is a very small proportion for which usual care is stated as a comparator without further information available in the research plan.

CHAIRMAN NORQUIST: Harlan Weisman.

DR. WEISMAN: Just ditto what everybody has said, it's fabulous, it really is. Just to supplement what Allen said, I think as we begin, as these studies begin being completed, having incorporated in something about reporting publications and classification of those would be equally valuable.

MS. FRANK: Yes. I'll just add that these taxonomy data are being combined with the rest of the data that we have around PCORI, so that we can get at end date and where the awardee is and their own dissemination plan. Absolutely.

CHAIRMAN NORQUIST: Freda?

DR. LEWIS-HALL: Congratulations, thank

you, and all that other good stuff. This is really, really great. As a follow on to Harlan's comment and to Allen's question, you proposed to share this. Might there be an opportunity to consider allowing others to import their study portfolio into this so we can really quickly identify what some of the continuous gaps are.

Right now, I'm not sure such a taxonomy exists that is widely available and easily useable, and if this is it, then that might be --

MS. FRANK: Yes, that's an excellent point. We are very much interested in understanding gaps comprehensively. Vadim has led the way in terms of using Inspire as a data visualization tool where we can pull in some other funders' portfolios into that. I would love to take you up on your idea to figure out how we can apply this taxonomy to those other portfolios. Absolutely.

CHAIRMAN NORQUIST: Christine?

MS. GOERTZ: Great, great job, Lori. I'm wondering to what extent is the Science group using

this in the cluster analysis? How are those two projects working together?

MS. FRANK: We didn't want to hold up the cluster work and teams around PCORI have already been thinking about the contents of the portfolio from a lot of perspectives, as you know. Now we have a consistent set of definitions that can be applied. Vadim has actually been key to pulling a lot of those analyses that underlie the cluster work and the whole portfolio analysis team. It is connected and will continue to be.

CHAIRMAN NORQUIST: Joe, do you want to make a comment here?

DR. SELBY: First, let me add my thanks to Lori and the considerable team here. It's going to be really great for us all.

I just wanted to say that we actually had the pleasure of reading a paper pre-publication that basically went to PCORI's website and went to the spreadsheet on our projects and did research on our portfolio already. We have one paper where people have already done that. In our comment, we

congratulated them and invited others to do the same.

With the increased capabilities that Lori has generated, I think mining the PCORI portfolio might become a national pastime.

[Laughter.]

DR. SELBY: We are going to adjourn for lunch.

CHAIRMAN NORQUIST: Yes, we are going to adjourn. It's on the second floor, but a different room than we were before. For those of you on the call, we are breaking for lunch and we will be back at 1:15 Eastern Standard Time.

[Whereupon, at 12:16 p.m., a luncheon recess was taken.]

A F T E R N O O N  S E S S I O N

[1:17 p.m.]

CHAIRMAN NORQUIST: We are live. We will restart now. We're not doing a count down, so there will be people on the phone now.

Welcome back. We're very pleased to have the next session, which is something that we have been trying to have at our Board meetings, which is a stakeholder perspective. This panel, we hope -- this particular kind of session, we hope, will be a regular feature of our Board meetings.

Today's panel features patients. The Board looks forward to hearing from you. Board member Gail Hunt will be moderating the panel. There's Gail up there. Gail, what I'm going to do is let you introduce the panelists, and we will let you take over after that as from a discussion point.

Gail Hunt?

MS. HUNT: Hi. This is, as Gray said, Gail Hunt, Board member. One of the things that we wanted for quite some time -- we have talked about

different stakeholders and bringing their perspective to the Board. Today, we have three people who have worked with PCORI or had PCORI grants, or they have been on panels for PCORI, and they're really representing the patients.

First of all, we're going to have Marc Boutin, who is the CEO, relatively new, well, maybe it's been a year.

MR. BOUTIN: Nine months.

MS. HUNT: Nine months; less than a year. Of the National Health Council. That's an organization that really brings together all the segments of the health care community to represent people with chronic disease and disabilities and their family caregivers.

Marc has been a leading voice for something called "Putting Patients First," which is an initiative of the National Health Council that is really quite focused on the issue of patients.

Then we're going to have Donna Cryer. She is a liver transplant patient. She has served on the PCORI Hep C Working Group that we have, and has

helped to refine the research questions for the TPSA. She's also been in a PCORnet Data Privacy Workshop.

She was the head of Cryer Health for almost a decade, which was a consulting firm dealing with strategic counsel, basically, to a number of organizations. She's a liver transplant patient, so she's actually experienced and going to be talking literally from the patient's perspective.

Last but not least, we have Celeste Lee, who is a 34-year patient of end stage renal disease currently on In-center hemodialysis. She has been a patient partner in PCORI research, and is involved with vasculitis, PPRN, which we were talking about the PPRNs this morning, and was on the PCORnet Patient Council. She has also spoken at a PCORI Panel on data privacy.

I'm going to ask each of them to have maybe six or seven minutes to talk to you about their perspective as patients and stakeholders, and then we're going to open it to questions.

Marc, do you want to start?

MR. BOUTIN: Sure. Thank you, Gail. I really appreciate the introduction and the opportunity to be before the Board today.

The National Health Council is an umbrella organization of patient advocacy organizations. It provides an united voice for people with chronic disease and disabilities. We work on issues that are important to all people with chronic diseases and disabilities.

Much like this organization, our membership is open to all organizations interested in health. We have the payer perspective, the innovator perspective, and the generic perspective, all in the organizations, but the governance is controlled by the CEOs of the patient advocacy organizations that you would all know.

We, like many of the patient organizations, played a role in the creation of PCORI, and in fact, we were successful in getting more than two dozen specific language items into the creation of PCORI. We're very, very pleased

and very active in the legislative process that brought this organization into being.

We have also been highly engaged in the implementation of your work, and I'm actually very pleased to say that I've been a member of PEAP, which I still chuckle when I hear that acronym. Patient Engagement Advisory Panel. It's been a great pleasure to serve in that role, and a great opportunity to help have impact on the work of PCORI.

I wanted to take a few minutes today and talk about the legacy of PCORI. I think you should be pride. You are five years out, but you have a legacy. A number of those items that I want to highlight include usefulness, something from a patient perspective that is so intuitive, and yet we fought in the legislative process to ensure that PCORI would be focused on research that was useful to the end user, patients, providers, policy makers, and we found it incredibly difficult in the legislative process, but we partnered with PCORI and that became a core value in everything you do,

and something that is of huge focus for the patient community and something we are truly appreciative for.

When you look at the whole concept of PCOR and what you have done with PCORnet, huge opportunities for a legacy. Perhaps most important from my perspective is the work you have done on patient engagement, meaningful patient engagement, and its impact not just on the comparative effectiveness research enterprise but the entire research enterprise. We are seeing dramatic changes. You are seeing changes even beyond the research enterprise that you should stand up and take a standing ovation for.

They include drug development throughout the life cycle. We're seeing innovators engage patients at the front end of drug development to understand what are the outcomes most important to them, and take that into the development process. We are seeing the regulators both here and abroad respond to that.

Huge, huge legacy in five years. As you

all know, the health care ecosystem is incredibly complex. I want to use the rest of my time to say that we need to do a lot more. You should be proud of your accomplishments, but moving forward, you have to enter the value discussion.

Everybody in Washington is trying to decide what is value. In fact, there are a number of models that have been put forward here in the United States about what value is. Not a single model has engaged the patient perspective in determining what value is.

It seems to me there is a lot that could be learned from PCORI in defining "value." Almost all of these models compound value with societal willingness to pay for the innovation. Two critically important issues, and I can tell you the National Health Council and the patient community is willing to have that conversation on payment, but they are not the same thing as value.

We need to collectively define what "value" is, and create some common principles around that.

From the patient perspective, we think of value in the context of having the meaningful opportunity to assess clinical outcomes, potential clinical outcomes, in the context of our own personal circumstances and our goals and aspirations. Something core to the work that PCORI has been doing.

If you think about it for a moment, and I remember using this example at your annual meeting you held jointly with AHRQ, my father was diagnosed with a terminal cancer. He was given nine months to live. He went through surgery, radiation, and eight rounds of chemotherapy. It extended his life by approximately three weeks. He spent nearly as much time in the chair getting chemotherapy as he lived longer. He lost half his body weight. He lost the dexterity in his fingers.

By the way, his sole source of income for the family was repairing antique clocks and watches. He lost his ability to generate an income. He died in an excruciating way, and it cost the system almost $900,000. That is not value

to anybody.

Imagine if we took the time to understand the personal circumstances of patients, understand their goals, and bake that into the delivery system. Think of two people with Parkinson's at the same level of progression of disease. One of them wants to ensure they can sleep well at night with their significant other and have a normal home relationship.

The other person wants to make sure their employer doesn't know, so they are not discriminated against in the workplace.

Those are entirely different outcomes and they lead to entirely different treatment regimens. Imagine the opportunity for PCORI to start to look at evaluating the impact of quality measures, payment models, delivery designs, that would help ensure that patients are able to articulate what their goals and aspirations are.

What is it in terms of the context of their personal circumstances. Are their goals actually met. Are their outcomes improved. Does

compliance and adherence improve. I'm getting the ax.

Let me go to my conclusion here very quickly, and that is that organizations survive when they are impactful and relevant. You have to look at how this organization can increase its impact and relevance in this climate where we know, and I can guarantee we are going to have a new Congress, a new President in 2017, that cost drivers in the health care marketplace are going to cause them to look at how we finance the system. You have an opportunity to use your resources, your leverage, your brand to drive the discussion of value, so that it has meaning for patients, and help reduce costs and give us a very new model of health care delivery.

I will say join the value discussion, be relevant, be impactful, and patients are counting on you. Thank you.

MS. CRYER: Today, I'm going to speak as a patient. I am a patient. I have chosen to self identify in that way, and to take on that identity

because it is the lens through which I truly look through to evaluate PCORI activities certainly, but really as I navigate life. That really is my bias, my lens, and my perspective.

As Gail mentioned, I am also the leader of a patient advocacy organization, the Global Liver Institute, which is an innovation and collaboration platform, attempting to create a transformative model of patient advocacy with liver disease as an use case, much like PCORI is trying to create a transformative model of research with many use cases that we have heard and that you have supported through your funding.

When I think of a patient today, because before I can get to my desk to act as a patient advocacy organization leader, before I can get to my phone to be a patient advocate outside of this room, I'm often known as "D.C. Patient" talking to online forums or speaking to those in the Twitter verse offering comfort and advice on navigation, I had to get out of bed.

As someone who is now four months after

joint replacements, I was thinking does rubbing my muscles with this particular type of cream before I start stretching decrease morning stiffness and the need for pain medication, and am I able to increase the number of steps.

I've been taking my morning medication, I'm thinking about does this generic version of the medication compare, have the same effects as the branded medication, and will look to my liver enzyme test to determine the results of that little experiment.

I am anemic and under the care of a hematologist, so as I consider my breakfast, I've been experimenting with the timing of caffeine with how I eat to increase iron absorption.

Throughout my day, as a transplant recipient and someone who is immunosuppressed, under multiple medications, we are thinking throughout our household about the effects of antibacterial soaps versus normal hand washing on a number of infections that I and my family members get throughout the year.

In terms of access, while I was waiting for my turn to speak, I was trying to access whether making an appointment on ZocDoc versus calling is actually a more effective way of getting an appointment, or trying to use an online refill system versus calling the system at my doctor's office actually resulting in getting a refill.

In the evenings, I spend reading medical journals, information online, various newsletters, and think how best to format and disseminate information in a way to a variety of patients in a variety of levels of health literacy and health backgrounds are able to take in.

At dinnertime, again thinking as an IVD patient, I have experimented with low residue versus gluten free diets. At bedtime, I'm running a little experiment right now to see if music versus reading versus aroma therapy helps with faster and deeper sleep, which I track on a wearable device.

I know as a patient what I have to contribute to the field of comparative

effectiveness research. Sometimes I wonder what doctors and clinicians have to offer that is equally as robust.

I say that only to think that patients have an intuitive sense of comparative effectiveness research that we have not previously captured, and many of the mechanisms that Sue outlined for you today and that Sharon did earlier this morning are so important for being able to take this experience and creating a discipline and specific mechanisms to translate these experiences and intuitive understandings into valid research protocols and clinically acceptable evidence and yes, higher keys of taxonomy, and we thank you for doing that.

No individual patient organization really has been able to do that. With my remaining one minute and 51 seconds, I will just talk a little bit about the evolution of some specific PCORI structures that have had a lot of meaning to me and to the patient community, and then end with some specific areas of emphasis.

As I looked at your milestone document, there were some that were included in there, but I think those that have been the most important to me as I saw an organization that was really struggling to set a tone and to figure out and define what patient centeredness was, I look at you home page today with a blog post by a patient who is transmitting her experience into expertise and her involvement with PCORI initiatives.

I look at the Board of Governors and the representation from patient advocacy organizations. Certainly, the development of your staff and the quality of your staff and Chief Engagement Officer, the Patient Engagement Director, and it was no coincidence that the first sort of first Chief Patient Officer in a pharmaceutical company came from the ranks of PCORI staff.

The advisory committees and committees' development in patient engagement, in particular, the rare diseases group. I went back and there is a fantastic and very robust, and could barely improve on the recommendations that were included

in the support of 2013 on transforming patient-centered research, building partnerships and promising models, so I would simply ask for people who have time to refer back to that, because it's a very rich source of patient-centered recommendations.

I participated as a merit reviewer, and I think the value and role of merit reviewers is really to explain and remind the other merit reviewers in some other disciplines what patient centeredness criteria is, and their ability in the moment to emphasize that is crucial.

The patient researcher match maker app challenge was a fantastic foray into the health IT, the innovation, stay in that space. What you are doing is essentially disruptive, own it. Be disruptive.

The last things I will mention that are really helpful is to suggest a patient-centered research plan, highlighting that option, and the specific document on how to write a research question. That along with the PCORI Ambassador's

webinars, workshops and roundtables offer opportunities for public comment, and have all been a fantastic demonstration not just to those who work for PCORI, but actually models that can be used and transferred to the rest of the health care ecosystem.

That is the true value of having created these tools and models for the rest of the ecosystem to use. I would ask you in the future to emphasize your support of the pipelines for patients, patient advocates and patient advocacy leaders, to participate in this work, to continue to train us, to continue to make sure to monitor the ratio of participation of patients and patient advocates on all types of committees, and to continue this cultural shift of patient -- not just as subjects -- patients as the researchers and your peers.

Thank you.

MS. LEE: It looks like I'm totally like a robot here, and I totally apologize. Can you hear me?

MS. HUNT: Yes.

MS. LEE: I took a fall like three weeks ago and broke my shoulder. I wish I could say it was something very dramatic, but it was a contact sport of making my bed, and I tripped over a pillow.

Anyway, as I was getting off the plane today, not wanting to put any pressure on this arm, I put it on this arm to get up and I popped something here. I'm like oh, my God.

Here I am, I am a patient for you. One who has the burden of illness but still wants to be engaged in the work that we are doing. I think that is the challenge that PCORI faces. That's the challenge that a lot of physicians face, depending on rare diseases, depending on certain burdens that the patient population is probably having to be able to participate, right?

For you, case in point, I actually get to show you what that looks like here today. It's a little bit of a challenge. That's why I was standing, thinking it might be easier, so now when

I get up a little bit later, it's going to be like oh, my God.

From the very beginning when the Patient-Centered Outcomes Research Institute was looking to come into play and we were looking at how do you define patient-centered outcomes research -- I've been very engaged with the National Kidney Foundation for 34 years, so we were one of the groups that wanted to participate and be one of the stakeholders, and try to help you all define what that meant.

I think so eloquently Marc and Donna have already described what that is. What is patient-centered outcomes research? It's really saying what is the quality, what is the value of the research that we're trying to get at that is going to improve the quality of the life of the patients and their caregivers or their partners; right? How do we actually do this better?

What I want to spend my time here on is talking about how do we do that better, meaning we have the research. I think in the United States

and I would even say within Canada and in Europe and internationally, we have amazing health services researchers. We have people who do amazing work. The challenge is how do we have them engage with the patients and how do the patients and their partners engage with them to actually add value.

We know they value kind of the sniff test, you know, when you see it, that it's adding value, but how do we actually, as Donna was wanting you all to perceive it, how do we get the patients prepared, trained, engaged in this type of work.

We know they want to, but do they, this is the challenge. We say we know they want to. A lot of patients have a very difficult time advocating for themselves. When we look at patient advocacy or patient engagement, you can kind of look at it at two different I would say cycles.

One is the private, the personal. This is how do I advocate for myself. How do I become an engaged active patient for my own health care, understand my own things, understanding what it is

I'm taking, understand how I make shared decisions with my physician.

That's been in research for a very long time, right. The folks at Dartmouth are doing an amazing job looking at shared decision-making models.

You have that type of advocacy, that type of engagement on the patient side. Then you have where they kind of cross over, so from the personal, they go to the public. How do they become a public advocate? How do they actually say now that I have this experience and I have learned what I have learned, I want to be able to apply that to a larger setting, to a much more of a public setting, where I can affect other people's lives.

That is what we are talking about at PCORI. We are talking about how people are taking their personal advocacy or engagement and then maybe jumping on a new track to do the public. I think that is crucial.

This chair really is PCORnet. You all

know about PCORnet. I've been engaged with PCORnet at two levels. One as a patient and advocacy counsel looking at privacy on a national level, and then I've also been involved with one of the patient-powered research networks, it is the Vasculitis Patient-Powered Research Network.

The reason I do that is the disease that I had that caused my kidney failure was vasculitis. It's a rare disease, and I was diagnosed at 17. I'm 50 years old, which is really awesome that I am. I'm very much involved in that research network.

One of the biggest challenges, I think there were 18 of them in phase one, but a challenge is saying if we're going to have these patient registries, we're going to create them, and we're going to have patients provide us the data, and I'm going to kind of not use my time to explain what PCORnet is, I'm really hoping you guys know what that is.

MS. HUNT: Yes.

MS. LEE: What the challenge was is how do

we get patients within our network, our patient population, who want to be involved with creating a registry, designing a registry, designing the web pages, looking at research, what research is important, how do we do that.

I'm going to get down to even the nitty-gritty. What I did for a profession at the University of Michigan is I oversaw patient-centered care for the health system. That was looking at how do we get patients engaged throughout our health systems, and not only at the point of care, but also within research and within education.

You have to go down to a really simple substructure, which is how do we orient them, right, how do we train them, how do we educate them, how do we define what "patient-centered" is, right. How you can help us, and then how do we translate that to physicians and the researchers to know how to do that.

It's all about setting expectations. It's all about defining and using the right language and

saying this is what we are really looking to do within the Vasculitis Patient-Powered Research Network. We actually want to have all the patients who are willing around the world to sign up and give us their information to try to collect as much as we can. How do we do that and why is that important? How will that help make patients who come after us have a much better outcome and better quality than we have had, if we can actually create and get this data.

We designed kind of an orientation. We basically gave them a really good education about vasculitis, again, why we are doing this, how they can help us, what this website is going to look like, would they be willing to be in an area of a patient advisory council, helping us with the design, look at the website, what type of design elements.

It is really about this whole training because the main thing with the patient-powered research networks, which I think is the future if we really want to do some amazing research that is

much more cost effective, right, is really being able to say you have all these patients who have data. All of us have data that we can give, right?

That data can turn into information based on how we get it and how we look at it, and that information is what is going to give us new knowledge. That knowledge hopefully will translate into how do we manage disease, how do we actually have certain types of socioeconomic patients who are suffering with various diseases, and how do we engage them. It will help us in different ways.

The most important thing that PCORI and PCORnet, and I know Sue and all the people are doing a great job, is we have to constantly be creating new ways of developing training, and what I call "patient development," patient partnership training.

It's not easy to be a partner. We don't necessarily know how to be a partner all the time. If we can actually draw on what my hope is, that PCORI starts to create a network that is a complete patient driven network, not patient organizations,

because what ends up happening is you end up with professionals and leaders who are patient engagement professionals. They aren't necessarily patients themselves, right?

If we can just have a network of patients throughout, and PCORnet could be one of the first examples of engaging all the patient leadership within PCORnet to help us create education and training networks that we can actually bring nationally to different groups around the country on how to best engage patients in the public sphere, not only the private, but the public sphere of advocacy.

Thank you very much. I'm out of time. Sorry.

CHAIRMAN NORQUIST: Thank you all very much. Do you want to make some final comments, and then we will open it up for other Board members.

MS. HUNT: Exactly. Thanks, Gray. I just wanted to start things off by saying I think this issue of the value discussion, which both of you really touched on as being a very important part of

what PCORI should be doing, you all think that, and I think we would kind of agree, and it kind of brings in what Celeste is saying, too, and that is how do we get to the patient outcomes, not just patient reported outcomes, because part of it is patient input, but get to patient reported outcomes that then translate to value. How should we be doing that in a way that is different from what PCORI is doing now?

MS. CRYER: I think that PCORI has set a tone, and set a certain floor for patient engagement and patient expectations as well as a specific model. No longer can various committees and agencies say that it can even be considered that they would have these discussions without having patients involved.

So, that helps get the foot in the door. Then being able to demonstrate through the different mechanisms we have worked with PCORI, with different stakeholders, that sort of takes away some of the argument about how patients wouldn't be able to participate in pharmacoeconomic

discussions and things like that.

Unless it is a medical intervention that is valuable to the patient, it really isn't valuable. Who else's priorities can creditably become ours? I think the work that PCORI has done has helped to sort of professionalize our advocacy, but also change the culture and expectations of other stakeholders on how they view and work with patients and patient advocates in a way that we can make much more progress in this value discussion than we would have been able to before.

MS. LEE: The only one thing I can say is I think PCORI work in tandem with the FDA and with other regulatory agencies and even NIH. I think it is really important that we have some patient reported outcome tools that have been tested, and that they very much have strong vigor, they can trigger -- I think that is going to go a long way in allowing us to do more work with patient reported outcomes.

MR. BOUTIN: The piece I would add to the responses you have received so far would be this.

The creation of PCORI in and of itself created a cultural ah-ha moment within the research community. Here's an entity that is substantially resourced, that is putting resources out into the community but saying you need to engage patients, you need to do it differently.

That changes culture. I think it was Winston Churchill who said "Culture eats strategy for lunch." What we are talking about are significant cultural changes. You had a huge impact on the research community, which is leading to the creation of some of these outcomes that are important to patients, but the data is only as good as it is used effectively in the delivery system.

We had the cultural ah-ha moment at the FDA when we mandated in the Prescription Drug User Fee Act number five that they had to hold 20 meetings with patient organizations. They have held 16 of them, and for each of the 16, the FDA officials walked away saying what I thought was most important to patients was wrong. All the research that led up to that application and

eventual approval of product was based on assumptions that were not focused by the patients.

What we need is a cultural ah-ha moment in the entire health ecosystem. In particular, where patients are receiving care, and I would draw an even finer point, for that 20 percent of people with complex chronic conditions that drive 80 percent of the costs, they are not getting access to the information that you are currently developing that will help them make that informed decision about what is the right clinical outcome for them given their circumstances and their goals.

We need to create that ah-ha moment, and one of the best ways you can do it is by entering that conversation of value, building out what a value model looks like in the United States. Something that is uniquely American. Something that is different. Something that engages patients, not to the exclusion of other stakeholders, but the opportunity for a new model done the PCORI way will take us to the place where we have that ah-ha moment, where we can then start

to realign the incentives and the models to drive this more effectively.

Huge opportunities, and in my mind, that is going to be your legacy, and that's going to be where you truly demonstrate lasting impact and relevance that will allow you to be a sustainable organization past 2019.

MS. HUNT: Ellen?

MS. SIGAL: I'm struggling for the right words, because I think I'm hearing similar messages but very different messages. You are disease advocate people that are dealing with a disease directly, and have a very different perspective on it. You know that because you know the granularity of your disease.

The value equation is important but it is also very granular. You mentioned the case of your father. I can give you examples of 15 or 20 maybe from last week of patients who have benefitted from treatments and lived to see a child's wedding or quality of life issues that were incredibly important.

The truth of the matter is we don't have the data. We don't have the data. The value equation is important, but without the real data on what is really important, without the PROs, without the tools -- we are doing this now. We're working on a label indication for FDA on PROs with the NCI. That should be easy. No. It's really hard because the disease model is different in every single disease and how you do it and generalize it is very difficult.

I think the other thing that I'm hearing, which I really agree with, is really framed patient advocates. People to really build that network. I can tell you we do these conferences all the time, and to get trained people, we keep on using the same old people because we don't even have a trained network of patients that can really speak up and who are frankly not intimidated by their physicians or not intimidated by sitting at a table like this and say look, I have a viewpoint and it's different than yours.

That is very complex, on trying to figure

out for us at PCORI how can we get granular enough to satisfy the needs of the patients, the real patients and what they want at different stages in their lives, and how we can contribute to that, and it's complex.

MS. LEE: It is. I'd just like to say I think what we are really talking about here is accepting risk/benefit, right. One of the projects I have been working on was really looking at trying to understand patients' preferences when it came to kidney disease, right, and how regulation happens in FDA.

To do that, the way we did that, you can't just go grab, contact all the kidney health organizations and say hey, send us 50 patients, we want to have this conversation, we want to try to assess risk/benefit, we want to hear what they are thinking, et cetera.

How we did that is we actually came up with an animated video that basically talked about what the FDA was looking at. We wanted patients at the table. We said if you are interested in this,

we're going to be doing these webinars, sign up for a webinar, different time zones, which basically were really user friendly.

They had patients on there that said hey, this is how the regulation works, this is the regulatory process, how devices get approved, drugs get approved. Gave it to them in really simple ways that they could relate to. Then we had a workshop that we invited 50 or 60 patients to come to have a much more productive conversation.

It was still a challenge. It was somewhat of a mechanism to say how do we get them interested to understand so we can really start to assess that risk/benefit.

As a 17-year-old female, my choices were very different and I made very different decisions than I would make now as a 50-year-old, right?

I think we have to build that into what it is that we are doing in risk/benefit. It is not going to be easy, and it is not going to be one size fits all. It's going to be from gray to black to white. It's going to go back and forth with the

same patients who at different points in their lives are going to assess risk differently.

MS. CRYER: I think your point is very well taken. My constellation of diseases is sort of a background in sort of the granularity discussion as well as how you generalize this. I think PCORI has a great role in terms of one, making sure that in these various formulas and algorithms there are patient-centered criteria that in some ways can be at a very sort of applicable level, and then in some circumstances, do need to be customized for the specific disease.

Just the fact of having them, to Marc's point about culture shift, and having a placeholder that is acknowledged that needs to be there for patients to contribute the elements of the algorithms is very important.

Then the emphasis on a basic level pipeline and CER training that can then perhaps be customized or added to by the different disease states, I think, is still an appropriate role for PCORI.

I remember serving at my very first FDA advisory committee meeting and realizing that it wasn't my training in the methodology of the data or having worked at a clinical trial recruitment firm, or actually having read the packet, unlike some of the other committee members, it was the fact that I had a legal background and that I was comfortable in that environment and comfortable speaking and integrating information and making hopefully a concise point.

I think there is a basic level of training for people who are doing CER in their daily lives and are not realizing it and labeling it as such, and helping them to translate that experience into comfort and confidence and participating in this at that first level, and then the advocacy organizations, I think, can help and support a partnership in taking it to that second level that is more granular.

MR. BOUTIN: Just very quickly, I want to respond. Ellen, I completely agree with everything you said. There is absolutely nothing that I would

take issue with.

I think it is "yes and," in other words, everything you said absolutely correct, there is no question about it. My greatest fear, especially when you look at the accomplishments that have happened at the FDA, and Friends of Cancer Research and you have been leaders in that. We have seen tremendous shifts in the biopharmaceutical sector where I believe we are going to get medicines in the very near future that respond to the outcomes of patients. That is huge.

What I am really afraid of in this current environment where we are defining "value" without patient input is we are going to develop systems where we don't get access to them or we are completely unaware.

When there are multiple treatment options that are available that have different outcomes that are relevant to patients, we should have a delivery system that helps us identify which ones are relevant to us given our circumstances and goals.

That was the point of what I was saying with my father. In his instance, and this was a number of years ago, less care would have been better care. For a lot of people with complex care, less care is better care.

In many instances, and we are seeing emerging data, it can provide huge savings to the system, which would allow us to have innovation in a way that responds to the needs of patients.

It's not one or the other. It's both. I completely agree with what you said.

MS. SIGAL: Push the companies and the relevant organizations to get more robust data so that when we have data, we can actually measure it. Right now, we just do not have the data. That is the complexity.

MR. BOUTIN: I completely agree with you again. The one point I would say is even where there is limited data, understanding a patient's outcomes, even at the end of life, allows you to structure their care in ways that are very different than we currently do.

Johns Hopkins, for example, has a great example where they deal with breast cancer primarily but also with other cancer issues, where they have incoming patients and they ask them what are their goals and objectives, what are their circumstances, and align care. Most people don't experience that.

DR. DOUMA: I want to thank all three of you. You have been doing great work for a long time. As people here know, I've been supportive of engagement for at least 25 years. I think it's core and critical not just to patients but to non-patients alike. We forget those sometimes.

I also agree, Marc, when you were talking about the need to prove value, and the way PCORI, I think, needs to prove value is by research, since we are a research organization.

My question to you if you could comment, what is the state of research and actually engagement causing better patient-centered outcomes, not input but outcomes, and to the extent that exists, what are the gaps and what can PCORI

do to increase that research?

MR. BOUTIN: Great question. I completely agree. You have to stick with what your core is. When I look at the portfolio of research, I'm seeing a lot of work on identifying issue reported outcomes, how to engage, how we look at the delivery system with shared decision making.

What I would like to see is us start to pull some of those aggregate individual pieces and bring them together, and do research -- I know you're doing research on systems and how they deliver care -- how do we evaluate systems more effectively to ensure patients' outcomes as they define them are actually being met. What are the impacts of that on outcomes, quality, and costs.

That is something that you can do within the spirit of the legislation. It's not comparative cost effectiveness research. It is what are the costs to the systems. I think there will be some instances where costs may go up, but I think there will be a lot of instances where costs will go down.

It's those sorts of data points that we in the patient advocacy community are going to need in 2017, when we have a new Congress, new Administration. We know we have a huge expense in access to care now, which is huge from the patient perspective.

We also know there are two million people with undiagnosed chronic conditions. We know from history and experience that as people enter the insurance market, they don't effectively use that access for three to four years. We're just getting to the third and fourth year. We're going to see the costs go up and it's going to create political pressure.

In this country, our tradition is we jack up premiums, we use more utilization management techniques, we increase cost sharing. That doesn't serve people with chronic conditions. If we can find an alternative way to root out waste, to provide care that is actually patient-centered, if the data shows that there is a correlation, we have a different model to bring to policy makers.

The data that you can produce along those lines helps us do our work more effectively to align with other stakeholders.

MS. CRYER: I would just add to that. Right now, to Ellen's point about the data not being there, it starts with the research question. Because prior to PCORI patients were not involved in the development of research questions, we got answers and literature and so-called "evidence" that didn't address things that we cared about. We also had end points that were not relevant to patients.

I think that the PCORI model and assistance and support for patient generated questions and patient researcher partnership co-development of what research questions are asked and the prioritization of that and development of the end points for those research studies will result in better evidence that can be included and plugged into guidelines and the normal sort of accepted evidence process.

There is such a big gap and we come up

with such a barrier to be able to sort of make our case in whatever venue it is and saying well, the evidence doesn't show, because the evidence wasn't created with patients in mind at the beginning.

Keeping that patient generated participation in the development of the question and the endpoints is, I think, PCORI's strength and value in what you can continue.

MS. SIGAL: We were just at a meeting with Rick Pazdur, an open meeting. He's the head of Oncology at FDA. He said that's what matters, not the end point. What matters is what matters to patients. This was a crowd of FDA people, NIH people, companies and patient groups. He basically said that's the end point that's important.

If we don't start to ask patients what they want, what is the end point important to you, and measure it. This is coming from the Director of the overseer. This is something we have to do really a better job on. I agree with you.

I can see the way we design clinical trials on the trials I'm working on, these were not

answering questions that patients were truly interested in. We have to really look at the entire enterprise to do something that would be meaningful for patients.

DR. JESSE: We have been looking at this for a while. What intrigues me is the classic equation for value is quality over cost, and what we have really been talking about here is the enumerator, because quality is in the eye of the beholder. That is the real challenge, to sort through that and engage a patient.

The interesting thing to me is that costs always seems to revert to dollars, and there is nobody in this country who does cost accounting that can actually really figure that out, for one. Secondly, the real costs is not always in dollars from a patient's perspective. There is a transactional cost. There is the emotional cost. There is a temporal overlay on this.

Now, it's no longer a two dimensional equation, it's a three or four dimensional equation. That becomes a real challenge to express

to people in ways they can understand it, and it's really hard to measure.

My point is don't ignore the denominator when we talk about patient involvement in that equation.

MS. CRYER: I absolutely agree. I think what is often left out in the denominator -- the cost is the caregiver taking off work, a lot of indirect costs, filling in for gaps in the health care system, whether they are paying out of pocket or caregivers and things. There are a lot of costs from the patient viewpoint that often are not included in many of the calculation algorithms. I think that number could be expanded from a more patient-centered view in the same way, but the quality being redefined to a more patient-centered view would add to the equation as well.

DR. JESSE: I think from the patient's perspective, if you want to summarize it in one word, it's either certainty or uncertainty, depending on how you are looking at it. My definition of "patient-centered care" is

personalized certainty. That is really what we are trying to do in PCORI, provide as much information to answer the questions patients want answered, what's going to happen to me, what's going to happen to my loved one. What are those right answers in a very tangible fashion.

That is very different than the whole construct of precision medicine. There are many, many more dimensions involved.

CHAIRMAN NORQUIST: I think we have time for about five minutes more here.

MS. HUNT: [Off microphone.]

UNIDENTIFIED: [Off microphone.]

DR. JESSE: My sense of precision medicine is it is very analytical, and it's taking things that we can concretely measure like a gene, like a biomarker. It does not take into account, at least from what I'm hearing, the social determinants of health. It doesn't take into account family dynamics. It doesn't take into account the impact on workforce issues, jobs.

MR. BOUTIN: It is the basis of what we

call the "chronic care trifecta," which is the clinical outcomes, which is where precision medicine can lend and can help you with some certainty on the clinical outcome, but the personal circumstances are where the social determinants of health lie, where geography lies, where all the aspects that impact your ability to take advantage of the potential clinical outcome or to assess which clinical outcome is best, and then the third component is while you are living, what's important to you.

Those sometimes run counter to some of the clinical outcomes that are thrust upon patients. We have to account for all three in order to get this right from my perspective. PCORI is in all three areas. I think there are opportunities to bring them together into a value that helps people to understand that, because I don't believe the rest of the world has had that ah-ha moment yet.

DR. JESSE: Sort of an example that kind of brought this home to me is for our patients in Alaska, we can provide state-of-the-art oncology

care in Seattle.

[Laughter.]

DR. JESSE: Because it's really difficult to do in Alaska, they don't have the extent of the health care infrastructure to do it. What is the disruptive factor of doing that.

MR. BOUTIN: I use a great example of my relative who lives in Northern Maine who has a serious chronic condition, has a child with autism, a mom with Alzheimer's who is living with her, single mom working part-time at minimum wage without a high school education, very limited health literacy and eight hours away from an academic medical center.

Do not tell me that each one of those factors does not impact her ability to access care.

MS. HUNT: [Off microphone.]

DR. LAUER: Thanks very much. I'm Mike Lauer from NIH. I really enjoyed all three of your presentations. One of the biggest frustrations of clinical research is that only a very small portion of patients with various disorders actually

participate in clinical research. Typically, the numbers are less than 10 percent.

There is also an interesting irony which is that for some common conditions like heart failure, many of the patients who are enrolled in large scale trials come from Eastern Europe. They're not coming from the United States. What are your thoughts about that?

MS. CRYER: I think your clinical trial needs to be in your pocket. The Global Liver Institute right now is working with Apple on a Hepatitis C study. When you look at cell phone penetration in African Americans and the low-income communities, it's very high. When you look at the American Research Institute information on use of cell phones or the Smartphone to search for interactive health information, it's very high, again, usually in medically underserved communities.

I think that one of the answers, I would say, is meet people where they are, sort of a general principle. I built a clinical trial

division. Meet people where they are as sort of the general principle, and right now, they are on Smartphones.

I think that the research enterprise needs to move there.

MR. BOUTIN: Three quick points. I think your precision initiative where you want to enroll a million people is going to have huge ramifications in terms of education, which is a huge step in the right direction.

Second point, involve us in the development of the clinical research. To Ellen's point, often times clinical trials are developed in a way that make them virtually impossible for us to participate, given our current lives.

I'll leave it at that. Thanks.

MS. LEE: I would absolutely agree with that. I think when you get the stakeholders who you want to have participating in the clinical trial involved from the very beginning of designing the right trial, then downstream, it's going to be much easier to engage the patient population,

because you have links to them. You have also designed it in a way that doesn't make it burdensome to the specific patient population. That is why I think it is really crucial to have them involved in the design part.

MS. HUNT: Marc, Donna, and Celeste, thank you so much. We really appreciate you coming and talking to us about how PCORI can utilize the patient stakeholder more. Thank you.

[Applause.]

CHAIRMAN NORQUIST: Thanks very much. Robin, are you ready? Next, we have a report from Robin Newhouse from the Methodology Committee, an update. There will also be consideration for approval of revised and new standards that you are releasing for public comment at this point.

We just need to be able to approve you to release it for public comment? Is that what the issue is?

MS. NEWHOUSE: Yes, that's correct.

We're proud to provide a report of the Methodology Committee activities over the past

year. David Hickam is here with me. David has been our chief partner on staff who has helped us through the review process for the current standards. It's hard to believe that these standards were presented to you in 2012, and were released for public comment, and then began use in 2013. Here we are in 2015, and it's time to review and revise our methodology standards once again.

That will be the major point of this presentation, to review the process that we use, give you a high level overview of our revisions, to review the process of review and voting with you, and then talk about the next steps in terms of revisions and release of the draft revisions for public comments.

We will be asking for you to vote for release of these revised methodology standards for public comment at the end of the presentation.

In terms of reviewing the process, this process used work groups to review the current methodology standards. In terms of the current standards, the good news is we have a lot of

experience with the standards in terms of public feedback and in terms of investigators using the standards to submit PCORI proposals.

The staff was pretty well informed about which standards they had questions about or which standards might use a little bit of tweaking or some revision in the language that was used to make it easier for investigators to apply those standards.

These standards were shared with the Methodology Committee, the revisions from the work group, which involved both PCORI staff as well as PCORI Methodology Committee members. They were then revised, brought back to the Methodology Committee over this past year at bi-weekly meetings. They were discussed, and sometimes there were multiple steps and repeated iterations.

At the point at which we were satisfied with the revisions, we had a face-to-face meeting scheduled on October 29. We all received a briefing book which reviewed our standard revisions and the draft standards. We all voted on the draft

standards, and any standards which had 11 positive votes were not reviewed at the face to face meeting. All standards that had some discrepancies or recommended revisions came back to the Methodology Committee at our face to face October 29 in person meeting.

At the end of the day, we held a vote for those proposed changes. There were a couple of standards that needed a little more work. Another subgroup worked on those changes. They came back to the Methodology Committee meeting on November 9 and we voted.

All standards that you have in your briefing book are the standards that have been revised and approved by the Methodology Committee.

In total, we made revisions to 25 standards. We combined some standards. The good thing about this review is we were able to look at the flow of the standards, and we were also able to look for redundancy across standards.

We did find there were some standards that could be combined to make them more logical. One

standard was deleted because actually it had some overlap with another standard. Nine standards were unchanged. Three new standards were added to the existing standard categories, either because they needed to be broken out for other reasons or because there was something we wanted to add to the standard to make it clearer.

There were five new standards related to designs using clusters.

For the most part, the rationale for any of the standards were really to clarify the language or to assure the alignment with the language that was being used within PCORI now. In some cases, it was to enhance statements that reflect the advances in methodology, and also to synchronize concepts across standards that were addressed.

This just gives you one example of a new standard that resulted in a standard process. This is from the causal model, causal influences. In this case, the recommendation was that we begin in Standard 1 by specifying that there needed to be a

causal model relevant to the research question specified.

That was just a brief description of the revisions to the current standards, but we also reviewed new standards, and those were standards on designs using clusters. You may remember that we hosted a work group of experts that came into the work group to make recommendations about a draft set of standards that were already drafted related to what is known about best practices in designs using clusters.

What you see before you are the five standards that resulted related to designs using clusters. I won't go into each and every one of them. They are in your briefing book.

Here are the standards. Once again, I will pause for a moment. Do you want Dave to make a comment here?

CHAIRMAN NORQUIST: [Off microphone.]

MS. NEWHOUSE: Okay. In terms of approval, our next step then is for you to make a recommendation that we can now post these revised

standards and new standards publicly. We will go through the same process as in the first set of standards. All public comments will be reviewed, processed, and categorized. Any revisions based on those public comments will be made to the standards. Those final standards will be brought back to you for approval before they are implemented.

I think the one thing I would like to add is the expected time line for feedback and revisions will be January through June 2012 [sic]. In addition, there will be a Methodology report revisions as well.

CHAIRMAN NORQUIST: On that last point, how long is the comment period open?

MS. NEWHOUSE: 45 days.

CHAIRMAN NORQUIST: All right. Revising and then getting it back. There wouldn't be another comment period, you would revise based on that and then put them out; right?

MS. NEWHOUSE: No. After we have the comments categorized and we address each and every

comment, we make the revisions to the standards, then we bring them back. There is not a second --

CHAIRMAN NORQUIST: That's what I mean. Let's have a motion to approve the release for public comment of the prepared new and revised methodology standards. If I could get a motion for that.

UNIDENTIFIED: So move.

UNIDENTIFIED: So move.

CHAIRMAN NORQUIST: I'll take one of those as a second. Now we can have a discussion. Any discussion? Harlan?

DR. KRUMHOLZ: I just want to make the point, first of all, how important this work is and how difficult it is to do, to bring everybody together and drive consensus. I wanted to start by acknowledging that.

Second, I just want to be sure that we are intently focused on the dissemination and application of this really good work. I've been on about the curriculum, and by not mentioning it, I'm not mentioning it again.

[Laughter.]

DR. KRUMHOLZ: I think we should be thinking about what software, what ways can we make this easy for people to use and to apply in study sections, in schools, for young researchers, and how can they find what they need. Right now, they are there, but I would love for us to invest as a group in some ideas, outsources, have a competition.

How can we take this body of information and figure out what the most efficient way to make it widely available and easy to use in ways that have not been done before. My worry always when I see this is this is really great work, it's not fully integrated enough into our application processes, not integrated enough into the dissemination piece so that students around the country and so forth can really leverage it.

You guys are the world's experts in this area. We have assembled amazing talent. They have been working with remarkable people in the PCORI staff. I just with the deepest feeling want to be

sure that we have done everything again -- it's all about the meta stuff today for me. Okay, we have done the main work, what are we doing around the edges of the funding, assessment, evaluation, and how is it being used ultimately so we can be accountable for not just having produced something that can be put in libraries, but actually something that will be used every day, what creative ideas, which could come from outside our field.

This is about talking to people who are experts in that sort of communication space. That is my two cents about saying I'm 100 percent behind this. I'm so excited that you have done this. I want to make sure it really becomes something that everyone can access, use, and integrate it into our work flows at every level.

CHAIRMAN NORQUIST: Allen?

DR. DOUMA: Really just a follow up on what Harlan was talking about. In our application process, how we do that will have a significant motivational impact on folks who apply to us and

therefore, presumably, other folks as well.

I was wondering if you could remind me of the two steps for the LOI and then for the application. There are 45 or 50 different standards in this document. Do our evaluators look at every standard and see whether or not that is covered under the application process at the LOI level or at the application level, and if not, is there any prioritization if number five is not there and number 20 is, that's okay? Can you talk a little bit about the nuts and bolts of how we actually carry this out?

DR. SELBY: David, you are in a better position than I am. Go ahead.

DR. HICKAM: Yes, thanks for that question. I think this is something that we have put a fair amount of effort into over the last two or three years.

First, in the merit review process, we have organized some materials to aid the reviewers, the merit reviewers, to refer to the methodology standards in evaluating applications for funding.

We have also instituted a process for those projects that move forward and are sort of on the launching pad for funding. There is a process by which staff evaluate alignment with the methodology standards, and essentially identify areas in which there may be certain problems for follow up. Those are directly communicated to the applicants, and we set up essentially a time line for them to provide revisions to their plans, having to do with the standards.

Now, we also have sort of a back end staff that we don't have as much experience with, which is to look at the standards again when projects are completed as sort of a preparation for that when there is actually a piece of the six month progress reports that do call out the methodology standards and any issues.

I think one of the good things about this is PCORI program officers are quite comfortable with discussing standards -- issues that are related to the standards when they have their follow up discussions with all the funded projects.

DR. DOUMA: Quick follow up on that. It's not the reviewers basically have a check off, here are all the 45 standards, this meets this standard, it doesn't meet that standard, and depending on what level the merit process is, it may get somebody calling the applicant or not. It's much more organic than that?

DR. HICKAM: Remember, there are a large number of methodology standards and reviewers kind of do their reviews based upon their own judgment about scientific issues having to do with an application.

That was largely why we put this second step in, for the projects that are moving forward on the path to funding, we deliberately put in a process to do the methodology standards review at that stage.

DR. SELBY: I have Leah and then Larry.

MS. HOLE-MARSHALL: Thank you. Thanks for the great work, always nice to see. It sounds like from the summary that it is mostly streamlining, more consistency, maturation and consistency with

other PCORI materials, now that we have had time to mature, related to these standards and changes. Is there anything that needs to be fast tracked because it is either an omission or something new that we have learned that we really want to plug a gap and that could be useful for funding announcements between now and six months from now? We might have to have a slightly different process.

I wholeheartedly support getting public input and making sure there are careful considerations, but sometimes we can do an interim process while we are finalizing that.

Is there anything from your perspectives that needs that kind of attention?

MS. NEWHOUSE: With the exception of the new standards, no. I would say it really was a matter of applying what we know works, and when the original standards were written, sometimes there was guidance and sometimes there was explanations, and it was taking the explanation and really refining.

Wouldn't you say -- David, would you

agree?

DR. HICKAM: I would agree. I think the major one is the standards on cluster designs, which we are seeing a lot of cluster designs being proposed for PCORI projects. Those are ones that seem it would be nice -- well, in fact, we are kind of referring to them already when we are evaluating those projects.

MS. HOLE-MARSHALL: I think in the PFAs that we are releasing, it would be good to mention that we have updated draft guidance and we would be looking at both and would welcome feedback, so that it's not necessarily mandated criteria that individual know and are aware of that, so that we are starting to incorporate --

DR. SELBY: We did that with the first round of standards, while they were posted for public comment, our PFAs referred to them and said something like you will not be held accountable for these but you are advised to check them.

MS. HOLE-MARSHALL: Correct. Should it pass today, I would suggest we do that.

The last one, we have talked a lot about these being kind of minimum standards to which we could all agree. Is there ongoing work for some of the more contentious or additional standards that we might want to look at beyond the cluster design ones?

MS. NEWHOUSE: Yes. We also discussed at our face-to-face nominations for new topics. We're going through a prioritization, as well as interacting with the Clinical Trials Advisory Panel. They are considering how these methodology standards can be used for clinical trials and what the gaps are as well, so yes, there is more discussion about what next. It's never done.

MS. HOLE-MARSHALL: Appreciate it. Thank you.

MR. BECKER: Thank you very much. Terrific work. On the curriculum, obviously people have created lots of curriculum, that all has to be updated. How does that happen, and is there funding needed to make that happen?

DR. HICKUM: Yes. It's a very good point.

We have developed a comprehensive curriculum based upon the current existing standards. My understanding is there will be some modest resources required to update that, since that was activity that was taken on by contractors.

MR. LIPSTEIN: -- they need to be updated? [Off microphone.]

DR. HICKAM: The CME, I think, has been released. My understanding is it has been released. The curriculum, my understanding is it is about ready, but we do need to align it with the changes in the standards.

CHAIRMAN NORQUIST: Alicia?

MR. BECKER: Do we have a date for release or an estimated date?

DR. HICKAM: This is an estimate. My understanding is they are going to be ready by about the end of the calendar year, which is only about three weeks away.

CHAIRMAN NORQUIST: Alicia?

DR. FERNANDEZ: Congratulations, this is a great document, and I can't wait to share it once

it is released to our fellows and our research training programs. I think it really walks people in a great way through thinking about the different portions of a study design.

I have a question for you which is about subgroup analysis. I noticed the document deals with subgroup analysis in different portions, including in the section on heterogeneity of effect. My question is was there discussion amongst the Methodology Committee in terms of how specific versus non-specific to be, and was that an area that attracted particular interest and discussion? It's certainly an area that as an NIH study section member we struggled with.

MS. NEWHOUSE: Yes. I don't remember that discussion in the Methodology Committee itself. In the subgroup, are you aware, David?

DR. HICKAM: There was quite a bit of discussion of that issue. We had working groups for each category of standards in which those kinds of discussions took place. A large part of the thinking about revising the standards was how

general versus how specific to be.

Generally, it was striking a balance. I think you will see in some parts of the revisions, for example, that new standard that has to do with causal inference, it really is actually upping the bar, it's pushing for more detail about causal inference models. I would say generally a lot of the changes did have to do with trying to push forward a bit in terms of more specificity and more detail.

MS. NORQUIST: The CME will be updated at some point.

DR. HICKAM: Just to make sure you understand, the plan is in place to update the CME modules to reflect the new standards. My understanding is the way those were designed, that's a fairly straightforward process. It's kind of like replacing the voice over and perhaps replacing some of the screen images on that.

CHAIRMAN NORQUIST: Harlan, is your tent card up?

DR. WEISMAN: Yes. I was reflecting back

on earlier when we were hearing about the PPRNs, patients being more actively engaged in research and generating research. By the way, I think the standards are great, and I'm really glad to see the new ones as well as the revisions.

I read something like the example that was given on specify the causal model underlining the research question and all the things that are being asked for within that, and I'm wondering how a PPRN would even understand that paragraph.

What are the efforts that are being undertaken, I guess, to not only bring the standard group of researchers on board with this and educate them, but also the atypical or non-traditional researchers whom we want to be participating and actively engaged in PCOR?

DR. HICKAM: I'm happy to answer that. Honestly, I think you're speaking to the importance of an interdisciplinary approach to research. If you have people who are less familiar with these concepts but are getting involved in patient-centered outcomes research, we should expect and

hope to facilitate that they have the right kind of partnerships with expertise that can help them through these issues.

We want there to be solid science in all of PCORI research, and that is kind of the point of the methodology standards. We just need to get the right people together.

DR. WEISMAN: What is our plan for that?

UNIDENTIFIED: I'm sorry. One of our fiscal year 2016 budget items that went through the EIDC was to develop a team science training module, so that would be one way, and also a separate training module for just patient participants as well. That is going to be happening over 2016.

CHAIRMAN NORQUIST: I think the key issue particularly in the patient module to translate it into English basically, kind of common language. Harlan, did you have anything else?

DR. WEISMAN: No.

CHAIRMAN NORQUIST: Okay. I don't see any other tent cards up, unless I'm missing them. Any final statements here, Robin, you want to make?

MS. NEWHOUSE: Just a couple of things just briefly to say how much the Methodology Committee is engaged in some of the other activities. You already heard about the PCORnet subgroup that is working on data quality and holding an expert meeting on December 10.

In addition, we are still working on new standards for complex interventions, evaluating multi-component, multi-level interventions led by Brian Mittman, more to come in that arena, as well as activities around the Patient-Centered Measurement Work Group that are working toward enhancing guidelines for incorporating patient reported outcomes in the electronic medical record.

I think the work on the Usual Care Work Group -- Hal, I'm going to turn to you -- is complete, and then will we do any kind of report back to the Board on the Usual Care activities. I know we just talked last week. I had a precursor of the end of the work.

DR. SOX: [Off microphone.]

[Laughter.]

DR. SOX: This is Hal Sox. I'm the Acting Chief Science Officer. The SOC approved the Usual Care policy some months ago, which basically is if you want to use usual care as your comparator, you really have to work hard to convince -- a single identified comparator. If you decide to use usual care, then you have to measure that usual care in every patient and in really both groups but specifically in the usual care group. That is our policy.

We are working on an article that reviews what has been said about the topic of usual care and plan to write that up as sort of a rationale for our policies, sort of turning the usual thing on its head and having the policy first and the rationale afterwards.

MS. NEWHOUSE: Thank you. I think the last topic was one we have already covered in terms of the dissemination activities, the academic curriculum and the CME initiative. Certainly appreciate the comments about how to advance dissemination of these activities.

With that, I will close.

CHAIRMAN NORQUIST: Okay. Thanks. Since we have a motion and a second, I just need a voice vote. All those in favor?

[Chorus of ayes.]

CHAIRMAN NORQUIST: Anybody opposed?

[No response.]

CHAIRMAN NORQUIST: Does anybody abstain?

[No response.]

CHAIRMAN NORQUIST: Okay. It passed. Christine, you have more time than you may need, I don't know. The next item is the revised Selection Committee Charter. Christine Goertz is the chair of the Selection Committee and will present this.

MS. GOERTZ: Thank you, Gray. I actually believe I have been asked to present this as chair of the Science Oversight Committee, although I am also the chair of the Selection Committee. I just want to put my conflicts out on the table right from the very beginning.

There are five clarifications or changes that we are recommending to be made to the

Selection Committee Charter. The first is that the charter was approved by the Board in July of 2014 and I think it provided a very sound framework for the work that we have done over time. However, we are realizing there are probably some updates that would be warranted regarding more flexibility to respond to changing needs and cycles and to reflect the responsibilities of the committee a little more clearly.

There are really five areas where change is recommended. I believe there is a draft in your materials. Just really briefly, the first thing the Charter does is codify that the Selection Committee's charge is to make recommendations for funding the targeted funding announcements as well as the pragmatic and broad announcements.

The second thing that it does is it increases the number of members of the committee up to 10 before --

UNIDENTIFIED: There are slides here.

MS. GOERTZ: Do we need slides? The next is to change the number of members. Before, we had

to have exactly six members. Now, it says we have somewhere between three members and 10 members, and that Methodology Committee members may be appointed but Board members have to be a majority, and also the SOC chair is no longer required to serve on the Standing Selection Committee.

The reason for changing the number of members is -- a couple of reasons. First of all, one of the issues we have had in the past with six members is at times you have had difficulty with a quorum, just because people had conflict or we have had to have meetings over holidays that made it difficult for people who were members to participate. Wanting a little bit more flexibility with perhaps having more members.

There has been some discussion about what is the role of the Selection Committee in making recommendations to the full Board, and interest perhaps in more Board members having a chance to get more involved in Selection Committee processes. This provides an opportunity for Board members who might be interested in being involved on the

Selection Committee to actually do so.

The third is to provide more flexibility in assigning ad hoc members to the Selection Committee itself based on expertise or need for a quorum, and also some flexibility in providing ad hoc Selection Committee members, ad hoc Selection Committee meetings themselves.

For instance, if the regular Standing Selection Committee is not able to meet to talk about a slate of recommendations either because they may not have the particular expertise or there may be just too much of a workload, this Charter provides flexibility in appointing ad hoc Selection Committees that would be able to perform that role as well.

The SOC chair is no longer required to serve on the Standing Selection Committee. It also makes the chair term's more consistent with some of the other committees that are in PCORI.

Finally, the Charter clarifies the issues that the Selection Committee will consider as they make funding recommendations to the full Board.

You can see that the Selection Committee will consider several factors, including Merit Review Panel scores on scientific program staff recommendations, programmatic fit and portfolio balance, duplication of funded research, and then methodological concerns.

CHAIRMAN NORQUIST: Why don't we get a motion to approve?

DR. LAUER: So move.

CHAIRMAN NORQUIST: Thanks, Mike. Second?

MR. BECKER: Second.

CHAIRMAN NORQUIST: Larry. Now, we can have a discussion, questions, comments. Allen has his tent card up.

DR. DOUMA: Can we go back to a previous slide? This slide takes us back to what we were talking about, about the evaluation metrics. Lori was talking to us earlier. The question raises the issue of you are supposed to make selection based on programmatic fit and portfolio balance. Does that call in use of our database to figure out how to do portfolio balance, number one.

Number two, how are we going to define or know when we have reached portfolio balance? Is that going to be part of the Selection Committee's training? I'm not sure I could do it.

MS. GOERTZ: Leah is raising her hand. I can make some comments, too.

MS. HOLE-MARSHALL: We have been using the cluster analysis to date, which is the precursor to having the full data work completed, and we have requested to separate the broad announcements, which from the Selection Committee we are still blinded to, unless they are outside of score order.

This is primarily for the more targeted investments that are significant resource wise, and we want information about how it fits within our current portfolio. We asked the program officer to provide us with some information, and we will now be able to use basic data from the cluster analysis to see that.

CHAIRMAN NORQUIST: Isn't part of the issue you will have some investigator initiated in the broad and you will have the targeted, and part

of the portfolio balance issue is if we have a large targeted announcement, I don't know, Hepatitis C, and we have five applications on the broad, then one would say we really don't want to fund these five other applications that may have good scores but yet are duplicating what we are trying to do in the large things. That is part of the portfolio balance question, I think, in some ways.

Yes, anything that can help us understand what we actually have in our portfolio right now so we can look at that is very helpful, whatever that mechanism is.

MS. GOERTZ: PCORI staff already cue up sort of the pro's and con's of awarding any particular application, and part of that is the issue of portfolio balance. I think the tool that Lori and her team has put together will be really helpful in facilitating the discussions that we have around that issue.

CHAIRMAN NORQUIST: I think that does bring up the other bigger question of where do we

want to be in the next three to four years, and what are some of those things we really want to push. That is still to be determined. There may be some gaps we are still looking for, but if there is nothing in there that can fill that gap, that's a whole other issue.

Alicia, I think, was next, and then Bob Zwolak.

DR. FERNANDEZ: Thank you. I'm a little worried that we are in the post lunch phase.

CHAIRMAN NORQUIST: Yes.

DR. FERNANDEZ: Postprandial hypoglycemia.

CHAIRMAN NORQUIST: I hear you.

DR. FERNANDEZ: The reason that I'm concerned about it is in my mind, and maybe it's only in my mind as a member, as one of the members of the Selection Committee, what is before us is really a way to have people decide whether or not they feel comfortable with the Selection Committee operating in almost in lieu of the full Board.

In other words, in my mind, this is connected to does it really make sense for us as a

full Board to say oh, when we look at the slate, wait a minute, that third study, what is the comparator on it, you know, which doesn't seem to me has made a lot of sense.

What I think this partly reflects, and again, maybe this is only my view, is not only having the Selection Committee work more efficiently and achieve a quorum and have the necessary expertise, but also to allow people to feel like oh, yes, this is a fine delegation of activities, and when the Selection Committee comes through and says oh, we have studies one through five, to feel okay with it.

Christine, could you comment on whether it is only in my mind that these things are linked, and for those of you who are on the broader Board and not on the Selection Committee, were these things to be linked, is this a good revision of the Selection Committee Charter? Does this feel appropriate, inclusive, correct?

MS. GOERTZ: I think you bring up an important point that we share and obviously some of

these changes were made in response to the discussion that the full Board had at our last meeting about what is the role of the Selection Committee, what is the responsibility of the full Board, and that is part of the rationale for increasing the number of Board members.

I believe it would be accurate to say that this charter assumes that the Selection Committee would continue to have the majority of responsibility for carefully vetting our grant applications and making recommendations to the full Board.

This Charter is not recommending a change in what is our current procedures, but what it is doing is it provides more flexibility so that more Board members who really are concerned or would like to be more involved, I should say, in how our applications are selected and recommended to the Board. It basically provides those people with an opportunity to sit on the Selection Committee.

CHAIRMAN NORQUIST: They are perfectly linked. It is linked that way because the issue

has been always what can we do in this Board meeting, in this room, in a public session, with approval of grants, and we are going to talk about how that is a fiduciary responsibility, but the issue of if you really have a concern about the quality and what's happening, and we need to have a process, the process is the Selection Committee, but we need to open it up basically so that those Board members who really don't feel comfortable having a general vote of just as a fiduciary issue need to get engaged at the Selection Committee. We have expanded the number who can be on there to open it up for those who feel that way.

The other issue is quite honestly, it was a practical issue, that there was too small of a number and we were having too many recusals and stuff as the Selection Committee members got into a little more detail, so that impeded some ability for the Selection Committee to function, quite honestly.

Barbara?

DR. McNEIL: Two comments, and the first

one is I agree with these responsibilities by and large, but I have one little caveat. Suppose a really critical question came up for which there was a well designed study that actually tipped the portfolio out of balance, so we were now putting in lots more money in one area, neurodegenerative disease. I'm making something up. I have no idea what it would be. Some neurodegenerative disease where we already had lots of stuff in that area.

Wouldn't that be okay, that makes me think the portfolio balance shouldn't be a dominant factor in some of the criteria, in our criteria, if we actually have excellent studies that come in that were answering critical questions that are in an area that may already have some rich set of studies in it.

The second question is however we accept this or whatever, if this Board agrees with those criteria and if the Selection Committee has made its judgment on the basis of those criteria, why do we have to review the grants at the Board level, because we have been talking about trying to speed

up the process.

CHAIRMAN NORQUIST: We need to at least approve the outlay of the funds, as a fiduciary. The other issue that may come up is you may come back as the Selection Committee and say to the Board, in this example of a neurodegenerative disease, you know, we would like to put an extra $20 million in of where we are over for this reason. I think then you're asking the Board from a fiduciary responsibility to say yes, we believe that's a good expenditure of the funds in that direction.

That is what we are really voting on as a Board, not on the actual whether this is a good grant or not.

DR. McNEIL: If I could just push that one step farther, suppose that was not the case, suppose all of the grants that the staff brought forward that had been approved by the Selection Committee didn't require any additional expenditure of funds, just pretend.

What is the marginal gain of this Board

saying I approve?

CHAIRMAN NORQUIST: You are still -- there is a new expenditure. Every time you bring in a new group of grants, there is a new expenditure of money. Do you see what I mean? Every time, it's like an outlay. We are just simply approving that outlay.

I'm going to exaggerate now. We could say, you know, we don't want to spend any money this cycle on grants, we want to spend it all the way over here on some other area or something.

DR. McNEIL: All right. This could be part of another discussion.

CHAIRMAN NORQUIST: Yes.

DR. HICKAM: I think there is actually a statutory issue that comes into play, too, to the extent that the statute provides the authority to this body. It becomes an issue of if this body tries to delegate its legal authority to another entity that doesn't constitute a quorum and it isn't subject to the open meetings and that sort of thing.

That is why I think it ultimately needs to come back to this body.

CHAIRMAN NORQUIST: Yes, that's true. On some level, you want the whole Board to be aware of what is being expended, even if there is a statutory. Bob?

DR. ZWOLAK: I was just pleased to hear the answer to Alicia's question. Mine was exactly the same as hers, and if I understand the response, people who feel the obligation to look more into the details of these individual grants can volunteer to serve on the Selection Committee.

CHAIRMAN NORQUIST: Let me be clear, volunteering to be on the Selection Committee does not mean you show up at one meeting and never show up again. That means participating on the Selection Committee. This group is really doing hard work, and I think that is important. Harlan?

DR. KRUMHOLZ: Here's a friendly amendment to that idea, which is it may be there are particular grants for which there is a reason that someone wants to raise it to the Board level, and

they can't either put in the time or they aren't on the Selection Committee.

I think there should be an ability for that information to come out in advance, someone to go to you and Joe and say I just wonder if we could talk about this. Just for the Board, there is the freedom to say on a routine basis, we should scan, it's our responsibility to look, and we should do that ahead of the meeting, so it's not just being presented but there is some ability to say here's what we are going to be voting on.

If somebody wants to pull something out to talk about, in that pulling out, there might be an opportunity to engage with either the chair of the Selection Committee or somebody else, which would resolve it before it got to the Board, or someone says I think this is raising a larger issue that we should be talking about, whether it is about balance or something else, that there is the capacity for someone, as there is for virtually any topic, to come to the chair and say do you mind if we spend a little time just talking about this.

CHAIRMAN NORQUIST: Right. I would just like to have a process where that was done in advance, so there was a clear discussion with the Selection Committee who probably had had a lot of this discussion, so we didn't take it up here because we run into some very difficult issues if we start exposing a particular grant in this open session so to speak.

DR. KRUMHOLZ: Yes. It could be resolved before it comes here, but any Board member still has the opportunity to say I want to talk about an issue or something.

MS. GOERTZ: I think that would be more of an SOP than an issue for the Charter, wouldn't it?

DR. KRUMHOLZ: Absolutely.

CHAIRMAN NORQUIST: Yes. Bob, Jesse, and then Rick, and then Mike.

DR. JESSE: Mike was raising his hand, too. I'm probably going to defer to him for the answer. At NIH Council, we don't vote on individual grants, but we do have to approve grants over a certain threshold, grants that go to foreign

entities, and there is a couple of other things.

It can usually be done as a bloc vote, and then if it does have to be discussed -- all of that is not done in an open session. It's done in a closed session.

CHAIRMAN NORQUIST: Right, very different for NIH, their process. Their advisory council is not open, it's not a public session. That is what is different about us. I think we need to remember the difference there, and they do have an open session where they hear about other things, but when they vote on the grants, it's completely not in a public session.

UNIDENTIFIED: Ours have to be open here at PCORI? Does it have to be open?

CHAIRMAN NORQUIST: Yes. For fiduciary responsibility, it has to be open.

MS. GOERTZ: I would argue that actually the work of the Selection Committee is similar in a lot of ways to the work of an NIH --

CHAIRMAN NORQUIST: Yes, let me be honest, that is what we were really trying to create here,

kind of a mini-advisory council that is not public, but then the overall Board votes on that part. Rick?

MR. KRONICK: In response to Harlan and then a question. Harlan, if any of us see a name of the grant that seems problematic, having some ability to discuss that ahead of time makes sense. It is the kind of one-sided view, that we don't get to see the things that are not funded, so it might help a little bit.

My question is it would be useful for me at least to have a sense of to what extent we have grants funded out of priority score order, and that might be information that has been presented but I don't remember.

CHAIRMAN NORQUIST: It is usually presented to the Board, we know that occasionally. Christine?

MS. GOERTZ: I would say that in every round there are a very small handful of applications that are not in priority score order or we skip over some applications that have a

better score to fund something with a little bit lower score because it's of such high programmatic interest to PCORI, or on occasion, we have skipped over a grant that had a very big score again because it was a lower program priority.

I would say when we do go out of priority order, that is the majority of the discussion that the Selection Committee has, those applications that are not in order. I would say most of our applications that we fund are in priority score order but it is also common for there to be a few that are not.

MR. KRONICK: A histogram, if you look at like percentile, you know, what extent are grants funded out of score order.

MS. GOERTZ: We can put those numbers together. I have never seen it across all the realms.

CHAIRMAN NORQUIST: That's a good idea. Allen?

DR. DOUMA: Two things. One is a comment, I apologize. In the document, it talks about in

one case "any standing committee" and in another case, "a standing committee." It is an edited change to "the standing committee" in a number of places. Just need to do that to make it consistent.

The question I have is in looking at the portfolio balance, do we consider the dollar value or the number of patients covered or simply the disease state itself?

MS. GOERTZ: Portfolio balance is a relative term. You saw earlier today the number of applications that we funded in different areas, such as mental health, for instance, is where we have the largest number of funded applications. You see as you go down, those are not in balance. We are not trying to fund exactly the same dollar amount or the same number of applications in an area.

Portfolio balance is really a pretty relative term. Where we have discussed it in terms of the Selection Committee really has to do with times when we have -- one instance that really

sticks out in my mind is we had two applications that had a similar score, both on a similar topic, but we felt that was an area where we really only needed one application in that area, not two that were somewhat duplicative.

We chose to only fund one in the interest of portfolio balance. That is more of how that comes into play rather than trying to really balance our portfolio in the way you might be thinking. Does that make sense?

CHAIRMAN NORQUIST: Join the Selection Committee.

DR. DOUMA: I understand what you're doing. I think portfolio balance, particularly in the last three or four years of our existence, is probably more important than it has been.

MS. GOERTZ: Right.

DR. DOUMA: Maybe it ought to be elevated a little bit more.

CHAIRMAN NORQUIST: Also, I think that is part of the discussion that we had earlier about we should know at the full Board what overall we are

spending our money on, and I think those are the bigger questions for the Board to think about. Maybe we have too much in a given area. Not to the specifics of an individual grant.

MS. GOERTZ: I would argue that is actually an issue for our research funding strategy rather than for the Selection Committee.

CHAIRMAN NORQUIST: Right; exactly.

MS. GOERTZ: I wouldn't try to address it with this Charter.

CHAIRMAN NORQUIST: No more tent cards. We can do this on a voice vote. All those in favor?

[Chorus of ayes.]

CHAIRMAN NORQUIST: Anybody opposed and anybody abstaining?

[No response.]

CHAIRMAN NORQUIST: Okay. It passes. Instead of a 15-minute break, you get a 20-minute break. We start back at 3:30 Eastern Standard Time for those on the phone.

[Recess.]

CHAIRMAN NORQUIST: The next topic is Lori Frank -- back again with Laura, and this will be an update on the merit review analysis. Laura, are you going to do it by yourself?

MS. FORSYTHE: I am.

CHAIRMAN NORQUIST: Okay, Lori is taking a break. Fine. You are free now.

MS. FORSYTHE: Thank you very much.

CHAIRMAN NORQUIST: Laura Forsythe, for those on the phone. I realize I didn't say your whole name. Okay.

MS. FORSYTHE: Thank you. It's been a real pleasure to provide you with regular evaluation updates, and the focus of today's topic is our merit review score analysis. I hope you all feel revived and energized for a very interesting discussion.

What I am going to do is first give you some background about the evaluation of PCORI merit review more broadly, and then I'll share some specifics on what we have learned to support ongoing assessment of our merit review related to

impact of the in-person discussion, as well as prediction of funding decisions and final review scores.

The first thing I want to do is recognize this work really started with efforts from Rachael Fleurence and other folks at PCORI when an analysis was done of PCORI's very first review cycle, and that work was published in the Annals of Internal Medicine in 2014.

There are two main things that paper reported on that I want to remind you about. The first is that agreement among different types of reviewers on how they scored their proposal was limited coming into the discussion but improved throughout the course of the discussion.

The second is that we showed that PCORI funded a different set of proposals having used a two-phase review that involved patients and stakeholders in the second phase than would have been funded if we used only one phase of review with only scientists.

This paper provided some important

learnings, and also highlighted some necessary future direction, including to replicate the findings with additional cycles of review, as well as to answer some additional questions like to what extend is there emphasis on technical merit in a review that incorporates patients and stakeholders along with scientists.

The other thing that I want to recognize is the contributions of a special work group that we put together for this effort. We had the pleasure of having Christine Goertz and Mike Lauer, as well as some of our science program directors, Yin-Ping Chang and Steve Clauser, and our PCORI evaluation and analysis staff, to come together to provide expert input on this work.

They helped us to decide what were the appropriate research questions and the right message to answer those questions. They helped us interpret the findings and to consider the implications for PCORI, as well as to plan out our dissemination strategy.

Before we go any further, I think it is

important to remind everyone who is listening what makes PCORI review unique. The first thing, of course, is that we involve and bring together scientists, patients, and stakeholders in review, so every proposal is reviewed by one patient, one stakeholder, and two or three scientists, and they first provide a detailed written critique of the proposal, and then we bring together all of those people to discuss the highest scoring proposal.

We ask them to bring to the discussion their own unique views as well as to listen to and consider the diverse perspectives of the others in the discussion.

The second thing that is unique about PCORI's process is the review criteria. In addition to impact of the condition and technical merit, PCORI includes the potential for the study to improve health care and outcomes, as well as patient-centeredness, and the approach for patient and stakeholder engagement as review criteria.

The scientists score all five of these criteria. The patient and stakeholder reviewers

are invited to score all five, but are required to score the three that you see in bold here.

PCORI set up its review process in this way with this level of stakeholder involvement because PCORI believes that research is most likely to have an important impact on patient health care if all of those people, the patients and the other stakeholders are involved at every step of the way, including in reviewing proposals, but the level of stakeholder involvement that we have is very unique, and there are some unanswered questions, like how do you balance the perspectives of all these different views at the review, and to what extent is technical merit provided in the review process.

The work I'm going to share today is one way that we can look and see how well PCORI is doing in going about achieving what we set out to do in terms of integrating these different perspectives on these unique criteria.

We set out to answer some questions including what is the impact of the in-person

discussion on reviewer scores and on agreement between reviewers, which criteria contribute most to the prediction of our funding decisions and final review scores, as well as to look at the relationship between the merit review criteria, including by reviewer type and over time, and to better understand what reviewers think about our process, such as the relative importance of the criteria and how well they work together in the process.

Today, I'm focused on the first two questions, but we are happy to provide more information about any of these at any time.

Just as a reminder, the merit review score analysis is just one information source among many including a regular survey of reviewers, focus groups of reviewers, and a number of other efforts that help inform PCORI staff and committees about how well we are doing and ways we can look for opportunities to improve.

The last thing I want to share before we get into the findings is that we are working on

sharing what we have learned from this. We have already shared our findings with PCORI staff and committees, as well as at a session at the PCORI annual meeting, and we are preparing scientific manuscripts.

Let's get into the findings, and we will start by giving you a preview of the important learnings. The first is that the in-person discussion has an important impact on merit review. You will see that all types of reviewers change their score through the discussion, and that leads to greater agreement after the discussion, such that PCORI brings together reviewers from a variety of perspectives that at the end of the process tend to come to agreement on which proposals best meet the PCORI criteria.

Secondly, we learned that all reviewers' impressions about potential to improve health care and outcomes, as well as the scientists' views on technical merit and the patient-centeredness of the research are critical to success in merit review.

We also learned that all reviewers' views

influence PCORI funding decisions and each other's final review scores, and that scientist reviewers have a strong influence on scores, and lastly, technical merit is important to all types of reviewers.

We're going to look first at the impact of the in-person discussion on reviewer scores and agreement between reviewers. First, I want to share that when we continued the analysis that we did in the Fleurence paper, we found that in the subsequent six cycles of merit review, in fact, we replicated the finding that reviewers have less agreement before the discussion than they do after the discussion, and agreement across reviewer types in particular improves on proposals that were originally scored in the middle of the scoring range.

We can drill a little deeper to try to understand a little bit more about what's happening in the discussion, so when we look across all the preliminary scores by every reviewer on every proposal, from the six recent review cycles, we see

those scores stayed the same 47 percent of the time. The scores are changed by one point on a nine point scale about a third of the time. They changed by fewer or more points 22 percent of the time. When there is a change of score, the majority of the time it's toward a poorer score.

We also compared changes in scores between scientists and patients and stakeholder reviewers. Scientists change their scores 50 percent of the time, and patients and stakeholders change their score 56 percent of the time. Patients and stakeholders were a little more likely to show larger changes in scores of two or more points, and the mean change was a little bit larger for patients and stakeholders relative to scientists.

We also wanted to understand at the end of the day after the discussion how much did the different types of reviewers agree on which proposals best meet the PCORI criteria.

We looked at those proposals that ranged among the top 20 percent among scientists, and we saw that two-thirds of those also ranked in the top

20 percent for both patients and stakeholders. There were a small number of proposals that ranked in the top 20 percent for only scientists and stakeholders but not patients or only scientists and patients but not stakeholders. Only 10 percent of the proposals ranked in the top 20 percent for scientists alone.

What we concluded here is that the scientists, patients and stakeholders tend to agree about which applications are the top applications.

In summary on the first part of the findings, I will remind you that we found that the in-person discussion has an important impact on application review. All reviewer types changed their scores through the discussion, such that there is a greater agreement after the discussion, and ultimately, PCORI brings together reviewers from a variety of perspectives that tend to agree at the end of the day about which projects best meet the PCORI criteria.

CHAIRMAN NORQUIST: Before we go on to the next, so that we don't get too much information,

why don't we focus on this particular issue first, if people have questions or other comments. Larry?

MR. BECKER: How do you know that the group discussion, people exchanging their opinions, is a good thing?

MS. FORSYTHE: That's an interesting thought. I think what we are looking for here is -- I think it will become more clear as well through the second part of the presentation -- we brought the people together to bring different perspectives and to focus on different elements of the proposal and the criteria, so I think it's not always appropriate for people to change their scores. Some reviewers may have an opinion that best reflect their view on the proposal that they should maintain, but what we hear from some of the additional work that we have done, like the reviewer focus groups and open-ended feedback on the surveys, is reviewers with a different perspective highlight some element that wasn't clear to a given reviewer prior to the process.

For example, scientists tell us about

patient or stakeholder reviewers really helping them get a better understanding of the real world applicability of a proposal or the feasibility to implement the study in a real world setting.

CHAIRMAN NORQUIST: Mike has a comment or question. He's smiling.

DR. LAUER: I love that question. With an observational study, it is unanswerable. One of the criticisms of peer review as it is currently done is that a group takes over and they actually lead to bad decisions. On the other hand, one of the compliments about peer review as it is currently done is if you bring smart people together, they come up with multidisciplinary dialogue and conclusions.

What is the right answer? Some funding agencies do not have reviewers sit down together, so I've done reviews, for example, for MRC, in which I have written my review. It goes into the hopper. There are other independent reviewers. Then the agency makes a decision about what they are going to do with it. At no time did the

reviewers actually talk to each other.

At NIH, PCORI, what happens is reviewers sit down in study sections and they talk to each other. The only way you could answer a question like that would be to do a randomized trial where you would randomize proposals to either go through like an editorial style review where you have a central agency that makes decisions about reviews that are done in an independent and blinded way, and you do not bring the reviewers together to talk to each other, that would be one arm. The other arm would be the study sections.

I think while that is beyond PCORI to do, I think the kind of science that is being done here and the granular nature of the data that's being collected on peer review is fabulous.

What it does is it enables those kinds of questions to be raised. The fact that you are seeing there are changes in opinion on peer review tells you that is a question that is worth asking which you otherwise wouldn't know.

CHAIRMAN NORQUIST: Joe?

DR. SELBY: Mike really answered this very well already. I think two things. Number one, your question suggests that it might be really good to do that same pie chart on the pre-discussion scores and see what fraction of the studies that were rated high on all three going in before the discussion, how does that fall out, how much difference there is, to get a little bit at this probably a false specter of group taking over.

If you were a Board member, what would you rather have, a study where scientists, patients, and other stakeholders all agreed the study was likely to change practice, or one where one or two or the subgroups didn't. I think that's the more abstract level.

Our model really is one that we bring the range of stakeholders together and seek the studies that all find are important and likely to change practice.

CHAIRMAN NORQUIST: Kerry?

MR. BARNETT: Just very briefly. If I read that slide correctly, I think what it said was

that all the participants are about equally likely to change their minds following discussion. I think we should take some solace from that. I think it would be more concerning if, for example, all the stakeholders were changing their minds after hearing from the scientists or even vice versa. Then it would feel like it was kind of an one way march.

If they are all kind of being informed and all learning from each other, I think that is probably a good thing.

MS. FORSYTHE: What you are describing is consistent with what we hear qualitatively when we interviewed the reviewers.

CHAIRMAN NORQUIST: Rick?

MR. KRONICK: Very interesting results. I think you said this shows that having kind of different sorts of people in the room leads to folks changing their opinions, if I understood it correctly. I haven't looked at similar data from AHRQ. Mike, I don't know if you have from NIH.

Certainly, in our study sections, there is

some amount of change. I don't know whether there is any more or less change. We also have diversity of folks on our study review sections, but probably not as much diversity as you have here, and it would be interesting to know whether there is any more change in this environment than there is in either NIH or AHRQ's environment.

DR. LAUER: I'd love to be able to answer that, but we can't. The way they collect the data here and maintain the data, individual reviewers, and the characteristics of those reviewers, here at PCORI it is done in a more robust way than what we do.

CHAIRMAN NORQUIST: Barbara?

DR. McNEIL: Thank you for those data. I guess what I would like to see, I understand there has been little change in those two columns, but it strikes me that the mean or the median between the two groups might be quite different, so that a change of minus 2 to the minus 7 might be quite different from a starting point from the stakeholders and the patients than from the

scientists.

I would find those data more interesting than that particular slide. Do you have those data?

MS. FORSYTHE: Yes, we can look at information in a variety of ways including what was the starting points.

DR. McNEIL: That would tell me whether the discussion is actually flipping somebody from a fundable to a non-fundable or a non-fundable to a fundable range. These data, for example, the patients and stakeholders could go from a 7 to an 8 or 7 to a 6, and the scientists could go from a 3 to a 4 or 3 to a 2. I can't tell the difference from this slide. I think that is really critical. I would really love it if you had those data.

MS. FORSYTHE: Yes, that's great. We can drill down some more.

DR. McNEIL: The second question is -- maybe it's the next slide. I thought you said that 80 percent of the reviewers agreed on the top 20.

MS. FORSYTHE: There is about two-thirds

of the proposals that scientists ranked in the top 20 percent that were also ranked in that same 20 percent --

DR. McNEIL: Very same proposals?

MS. FORSYTHE: Exactly.

CHAIRMAN NORQUIST: Harlan?

DR. WEISMAN: I had a clarifying question, whether that was pre or post.

MS. FORSYTHE: This is after the discussion. There is a lot more to share, too.

DR. WEISMAN: I think discussion and debate among equals, people perceive themselves as equals, is healthy. Rich makes it more rich outcomes. There is no way of knowing here whether there is scientist versus lay-person intimidation. Again, how willing are the non-scientists to stand their ground if there is a debate?

MS. FORSYTHE: I think we saw that here, that there is no change in scores 50 percent of the time among scientists and almost 45 percent of the time amongst patients and stakeholders, and I think the most robust source of information on what

you're asking about is what we hear through our focus groups and our open-ended comments on the surveys where we have heard resoundingly that people describe a very balanced process.

CHAIRMAN NORQUIST: I think the bottom line here is what you do with them, are we getting what we want out of the process is really key. We just need to be sure about that, and some more qualitative kind of analysis can help you with an understanding.

All right. Next?

MS. FORSYTHE: Part two is looking at which criteria contribute most to the prediction of funding decisions and of in-person review scores. Like the data we just went through, I think this is well supplemented with our qualitative information, although the focus today is on the quantitative.

To look at relationships with our funding decisions, we first conducted a logistic regression model to predict funding status, yes or no, and the predictors were the preliminary review scores for each of the criteria by each of the different types

of reviewers.

We did this to reflect their views on the proposal that they brought to the in-person discussion. What we found is the significant unique predictors of being a funded proposal were the scientist ratings of potential to improve health care and outcomes, technical merit, and patient-centeredness, the patients' ratings of potential to improve health care and outcomes, and the stakeholders' ratings of potential to improve health care and outcomes.

There are a few things I want to highlight here. The first is that each of the reviewers' views are represented in some way in this model. The second is each reviewer's views of potential to improve health care and outcomes were a predictor in this model, in addition to the scientists' rating of technical merit and patient-centeredness.

What that suggests is that there is something important about each reviewer's view on that same criteria that is playing the role here.

The second thing we explored was the

relationship between these criteria scores and the in-person overall scores for each type of reviewer at the end of the discussion. To do this, we looked at a series of multi-variable linear regression models, one for each type of reviewer.

Looking first at the scientists, we found that the factors uniquely related to scientists' overall scores were the preliminary scientist reviewers' views on potential to improve health care and outcomes and technical merit, as well as patient views on potential to improve health care and outcomes, and stakeholder ratings of engagement.

When we repeated this model for patients, we found that the significant unique factors associated with patient overall scores were the scientist ratings of potential to improve health care, technical merit, and engagement, as well as the patient ratings of potential to improve health care, patient-centeredness, and engagement, and stakeholder ratings of potential to improve health care and engagement.

Lastly, we did the same for stakeholders, and the significant predictors here were the scientist ratings of potential to improve health care and outcomes, as well as technical merit. The patient ratings of potential to improve health care and outcomes, and lastly, the stakeholder ratings of potential to improve health care and outcomes, patient-centeredness, and engagement.

I want to show you all three models overlaid on one slide, and even though it provides a lot to look at, the reason I think this is important is to highlight that there are associations between every type of reviewer's preliminary scores and every type of reviewer's final overall scores.

Said differently, each box on the right here has at least one blue, one green, and one purple arrow drawn to it.

We think that suggests that all the different types of reviewers are listening to and are influenced by each other.

The next thing I want to highlight is the

relationship between the scientists' rating of technical merit and the final overall scores for each reviewer type.

The strongest predictor in each model was the scientists' ratings of technical merit. Lastly, I want to highlight again the importance of each reviewer's views on potential to improve health care and outcomes in these models, that again suggest there is something unique and additive about the way each of these reviewers are considering criteria.

When we are looking at that series of three regression models of overall scores, the main takeaways were for each reviewer type, final overall scores were related to criteria scores from all other types of reviewers, but the strongest predictor of final overall scores for every type of reviewer were the scientists' ratings of technical merit. Also, that assessment of potential to improve health care and outcomes by all reviewer types.

In summary on this section, I want to

remind you that we learned that all reviewers' impressions of the potential to improve health care and outcomes as well as scientists' views on technical merit and patient-centeredness of the research were critical to funding in PCORI review, as well as the fact that scientists, patients, and stakeholders influence spending decisions and each other's final overall review scores, and scientist reviewers have a strong influence on scores, and lastly, that technical merit is important to all types of reviewers.

I want to open it up again for questions as well as to direct your attention to some questions that we have for you, which include your ideas about other questions about merit review that we could answer with work like this, as well as other means to examine the influences of patient and stakeholder reviewers.

CHAIRMAN NORQUIST: We have five minutes. The better way to handle the second one is by sending you those questions. Rick?

DR. KUNTZ: Thanks. Just a technical

question, just so I can understand this. In your final models, were those independent variables in the final models, so you have a model of stakeholders as an independent variable, and potential to improve health care from the stakeholders and potential to improve health care from the scientists were independent?

MS. FORSYTHE: That's correct; yes.

DR. KUNTZ: That doesn't make sense.

MS. FORSYTHE: What it suggests to us is that there is some thing different or unique about the way the different reviewers are looking at that criteria that make it important from all of their views.

DR. KUNTZ: But it would go against your thesis that they were influenced by the scientists.

MS. FORSYTHE: Mike, do you have something to add? I'm not sure I fully understand the question.

DR. LAUER: What you can tell is -- by the way, they did this both by standard regression and they also did this using a regression which takes

away a lot of the assumptions.

You can't tell directionality. You can only say the associations are there.

MR. BECKER: Don't you think it's a little bit curious that the final model, which would be the independent variables, employed both, whatever, the potential to improve health, the stakeholders' view and the scientists' view were independent.

DR. FERNANDEZ: [Off microphone.]

MS. FORSYTHE: That's correct. The outcome is actually the scores among each reviewer group. I just want to clarify we're showing three separate models here but in one diagram so you can understand how the findings relate.

DR. FERNANDEZ: I think what that is showing is or the way I'm understanding it, tell me if this is wrong, Laura, is what it is showing there is for the patients' assessment of the potential to improve health, that green arrow going to scientists states that is a contribution to then the scientists' final scoring, and there is actually an arrow from the stakeholders to the

scientists showing that is actually influencing the final scores.

What we don't see and what we could present, and I would present, is how all of these load onto the last score, whatever you have. I think that is implicit in some of the things you put out, which is the scientists' scores is weighted out more, but I don't think we can see that in here.

On your point, does that make sense?

MR. BECKER: If you put all the terms on the left in the model and then you used the scientists, patients' scores in the linear regression model, and you have an independent model at the end, the stakeholder independent model included or did not include the potential for improving health and the stakeholders' potential for improving health and the scientists, to me that is just curious they could come out as independent terms.

MS. FORSYTHE: It did include both, and I think that is something that is really interesting,

that there is some unique variants prescribed to each of those different views on that same criteria.

UNIDENTIFIED: I think one of the main points here is that you have done a really nice job of fostering a culture of science, of peer review. I just want to say that.

CHAIRMAN NORQUIST: Yes, and I think that is really critical and it is also very helpful in some ways to understand what is actually happening.

DR. FERNANDEZ: Can I make one more point, which is there is this other review taskforce that Barbara and Bob Kaplan and Mike Lauer sometimes and I and others are on, in which we are looking at the entire review process. We would love to get input from folks. We have been heavily influenced by looking at the scores as it should be. Congratulations to you.

CHAIRMAN NORQUIST: How do they give input into that? To whom?

DR. FERNANDEZ: They can give it to me or Barbara.

CHAIRMAN NORQUIST: Who is in charge of that group?

DR. FERNANDEZ: I am.

CHAIRMAN NORQUIST: Okay, then it's going to you. It would be easier. Thank you very much. Harlan, if you could be quick.

DR. WEISMAN: It's a question and maybe an observation. I just found it curious that patient-centeredness was dropped out. There are so many lines. That patient-centeredness was not a primary predictor here. I was wondering what your thoughts were.

MS. FORSYTHE: It's a really important point, and I think it relates back to the way I introduced this, which is this is one way of looking at these questions that we have, and these models explain an important proportion but not the entirety of the outcomes here that we are talking about.

For that reason, we combined this with what reviewers tell us about their experience as well as we are undertaking a qualitative analysis

of the merit review critiques themselves, so that we can really better understand the strengths and weaknesses of the proposals we choose to fund and don't fund, as well as viewpoints the reviewers of each different perspective are providing on applications.

I think taken as a whole, we will be able to better answer some of those questions.

CHAIRMAN NORQUIST: Okay. Barbara?

DR. McNEIL: One final question. It strikes me it would be interesting to look at these data in terms of original submissions and resubmissions. They may differ.

CHAIRMAN NORQUIST: The other thing that may differ is over time as the groups change, too. It may change over time. If only human behavior were so predictable, it would make my job so much easier. There is the whole aspect of human behavior and when you get into groups, what happens that you are not going to rationally make some sense out of. Okay.

Is that it? Okay. Thank you very much.

The next item is workforce training proposal that Joe and Freda are going to present. Who is first?

DR. SELBY: I am.

DR. LEWIS-HALL: I'm listening.

DR. SELBY: Thanks, and thanks to Rick Kronick and Harlan Weisman. Harlan from the RTC has been championing this from the Board perspective as we talked about a possible role for PCORI in workforce training, and Rick from AHRQ has represented the fact that AHRQ is actually charged in authorizing legislation as being responsible for workforce training, they have a long track record in workforce training, and as you will see, they have a lot of interest in the particular topic we are going to cover.

This is an initial discussion, no real decisions. I think some sense of the Board would be valuable for us and in particular for the RTC going forward.

Here are some questions. Is research workforce training an appropriate area of

investment for PCORI? If you feel that it may be, is a specific focus on patient-centered research within learning health care systems a reasonable approach.

We're going to propose that it would be a good potential investment, or at least it's a topic area in patient-centered research training that bears observation and thinking.

Third, if PCORI does go in this direction, should it channel all of its work or any resources through AHRQ, we will basically say that makes sense to us, and fourth, does the time line seem reasonable if you support the first three.

Dating back to the first half of 2014, we had two meetings at the IOM with systems leaders. First, we met with the Chief Medical Officers, Chief Quality Officers, Population Management Officers, and Financial Officers, and then two months later we met with CEOs of health care systems, many of the systems but not nearly all were systems that contributed data to PCORnet.

Essentially what these systems said was

yes, we're sitting on a lot of data now, and we really don't know that well how to use it effectively. You are telling us research is important. We are telling you that the research needs to focus on questions that really matter to us, our own bottom lines, our ability to perform, our ability to improve outcomes, with the efforts we have put in place to evaluate the efforts we have put in place.

What we really need is researchers that know how to talk to us. Researchers that understand the language and the culture and researchers who don't propose to take our funds, go away for two and a half years, and come back with the answer, but instead, propose to sit down with us periodically and iteratively look at the data and learn from it.

A very different model of research, but it would be good to get people who know research methods involved in that set of activities. That was a clear message.

Bob Kaplan from AHRQ was in the audience

that day, and he was particularly struck, and I think it resonated with thoughts that were prevalent in AHRQ at that time, so very quickly after that meeting AHRQ expressed interest in conversations with us about expanding its already pretty substantial PCOR training programs to include a new focus on training researchers to work with learning health systems.

We have had several discussions at the RTC in the last six to eight months, and at least one or two of them were graced by the appearance of representatives from AHRQ, and that has only strengthened the thinking. I think the sense is, and Harlan Weisman, you could weigh in on this, but I think the sense is there is interest on the RTC in pursuing this idea that there may be a need for workforce training in this particular area, of training people to work in delivery systems, to actually move toward the vision of learning health care systems, and maybe in the process making PCORnet look more valuable to the systems in which it resides.

Meanwhile, in other sectors, at Academy Health, and I'll give you a little more information on this, they are always interested in workforce training, and they are mounting a panel to discuss future needs in health services research training that starts -- actually, it starts later this week.

The Robert Wood Johnson Foundation, and I am sorry Harlan had to leave before we got to this, but the Robert Wood Johnson Foundation has been very active in clinician training to participate in research. They have some new initiatives that need to be watched.

Lastly, the point is we could bring a novel focus to workforce training by focusing on training in this area, which has not really had a lot of attention yet.

AHRQ is actually going to convene in the first six months of 2016 a panel of up to 15 experts to advise on a roll out of the workforce training in this area. It will have panelists from learning health systems, health services research, PCOR research fields. It looks like it will take

six months for the planning and evidence gathering and implementation of the training program in the second six months of 2016.

Next, in our view, there are major advantages to collaborating with AHRQ if we go in the direction of wanting to put resources towards this kind of training.

Researcher training is already established at AHRQ. We would be hard-pressed and ill advised, we think, to set up a researcher training program inside PCORI, especially since the legislation assigns this responsibility to AHRQ and they do it.

PCORI can leverage AHRQ's expertise in this area of research training, and could after consultation with AHRQ about what resources they have to put toward it, decide in the Board's wisdom to augment those resources if they thought it was prudent.

Academy Health is launching its periodic, about every seven or eight year, reassessment of training needs in health services research. My understanding is that they have a strong interest

in this topic area, just not to fund it, but to weigh in on the need for it.

They will have at the table AHRQ, PCORI, the VA, and Robert Wood Johnson Foundation, so all the players are at this table that AHRQ is convening.

RWJ, the Robert Wood Johnson Foundation, has recently ended its funding of the traditional clinical scholars program, which trained physicians and nurses to do clinical research. Not a particular interest focus on the learning health systems or big data or electronic health record data at all. Pretty much a focus on preparing one's self to get NIH and AHRQ and RWJ grants.

The National Leadership Program is much more -- is a fascinating program, building a culture of health in the country to obtain the best health outcomes for society, strong focus on interdisciplinary collaboration.

There are four branches, each with its own site, at which the National Leadership Program initiative is being rolled out. One is called the

New Clinical Scholars Program. That is at the University of North Carolina at Chapel Hill. They have a much broader range of clinicians than the old Clinical Scholars Program had.

They train anybody within the health care delivery system, not just clinicians, but among clinicians, pharmacists and physical therapists and others, in addition to clinicians and nurses.

Their emphasis is not squarely and entirely on research any more. It is on using research. Some people will learn to do research but it's really on leadership. Some of the other programs are even more interdisciplinary. Some of them actually require that a team apply, not just one person.

These are not site-based firms. You don't go to UNC to take your training, you don't go to Minnesota or the other two sites. You stay in place and learn from a distance.

They expect to have 50 trainees in each of four programs, so once started, which will be by the end of 2016, they will have 200 trainees. This

will be in all topics, community based primary care and this new clinical research. None of it is explicitly focused on delivery system research, but any of them could be in that area.

We could send people, we could encourage people from PCORnet or elsewhere to apply for the funding from this program.

The National Clinical Scholars Program is what became of the former Clinical Scholars Program, so it is also in play here. When RWJ ended its funding of the Clinical Scholars after last year's awardees were named, the four host institutions, and let's see if I can name all four; Yale, Michigan, UCLA, and the fourth one --

UNIDENTIFIED: [Off microphone.]

DR. SELBY: Thank you. The institutions decided to carry on a version of this. This is still directed at physicians and nurses. It is funded by the institutions. I believe they are finding some other small bits of funding here and there. They will have about 20 scientists across the entire program recruited per year. Again, this

is not particularly based on learning health systems.

PCORI could contribute to this by promoting a strong emphasis on patient-centeredness, by focusing particularly on the learning health system and researchers who can work within learning health systems, and on possible synergies with PCORnet.

These are the three questions, introductory discussion, and I'm very glad Rick and Harlan are here to help lead the discussion, and I think our sense is really that it makes sense to go deliberately here until we get a little bit more of a shake out on what RWJ is doing and what AHRQ's six month evidence assessment leads to, as well as Academy Health's.

Thanks.

CHAIRMAN NORQUIST: Okay. We will open it up for discussion now. Sharon?

DR. LEVINE: One of the topics the Governance Committee has been considering in terms of Board development is an update for the Board, a

status update, a landscape review of the state of health services research. I think this fits very nicely into that, all of what you described.

I think one of the things to consider is that citizen scientists in some way includes clinicians who are not trained as scientists, so when we talk about citizen scientists or programs that look at bringing training to sites where care is being delivered and training cohorts of I would say patients, community advisory boards, and clinicians in that health system together, doing health services research in the health system is a model that might be worth exploring and certainly PCORI, I think, has a lot to add to a conversation like that. I'm not quite sure where it fits into all of these programs.

DR. SELBY: That actual notion of a team -- in one lengthy conversation with RWJ, I said it sounds like they are focusing many of their activities on the team, which could include a clinician and a member of the community and/or a patient.

I'm just not sure yet how much they are focused on the learning health system versus the learning community.

DR. FERNANDEZ: I'm very supportive of this. I think it would be fantastic for PCORI to contribute to increasing capacity in research. I'm quite familiar with the RWJ new call, as we thought about responding to it, and it is as you correctly point out not about creating researchers.

Many people were sort of worried about what will happen now that RWJ is going away for a research path, as RWJ has been a great way, particularly for non-traditional researchers, to enter research.

I think it has really given people a step up in the ability to carry out research. I think when I see this, I love the idea of further investigating it, love the idea of doing it through AHRQ. I am less enthused with the idea of doing the learning health system, because I think maybe I don't understand it well enough, but I'm not seeing -- it sounds a little bit too narrow to me.

I guess what I don't understand well enough is the other elements of the AHRQ portfolio in which people could then apply. I also don't understand and it would of course yet to be determined would this be the sort of traditional K Award model, would this be more similar to RWJ or the other attributes of this that would make it a different sort of program, other than saying the research needs to be focused in this area and so on.

From my perspective, this is a big green light, but I would love to know more over time as you work through some of those issues how you are going to be thinking about this.

CHAIRMAN NORQUIST: Steve?

MR. LIPSTEIN: Joe, I wanted to comment on the kind of workforce we're talking about training. What stimulates this is for those of you who have worked at Kaiser for a long time, you all have been combining electronic health record data with claims data for a long time, but the rest of the United States is just now catching up as health systems

are being put on this pathway to risk in their new payment models.

They are combining both claims data and electronic health record data into these huge datasets. What they lack in all of their health systems are the data scientists who essentially know what to do with this, so there is this new cadre of expertise that is evolving in our industry along with software to go with it, how you query those large datasets to do risk stratification, to do disease registries, the things you all have been doing for a long time.

None of us have really thought about how we would use those same datasets to do outcomes research. I'm not even sure we have even thought about how you standardize the data elements inside these datasets to measure outcomes, because typically, once the encounter is over or the episode of illness or injury is over, the data becomes more limited in terms of longitudinal data on outcomes, especially if you don't have a continuing care relationship with the patient.

The reason I went into that little description was when you think about workforce training for PCOR, it's not just physician scientists or clinician scientists, but it is also data scientists, and you put them up as non-clinicians, but this whole area of data science, and what are we going to do with these new datasets we are creating for other purposes, and how can they benefit outcomes research, it seems somehow to be part of this discussion.

CHAIRMAN NORQUIST: Let's go to Rick and AHRQ.

MR. KRONICK: Thanks. Alicia, in response to your question, we have a variety of training programs. We have institutional K-12 awards. We have individual K awards. We have had K-32 awards that are not PCOR specific but have PCOR pieces.

These training programs have been focused on training people to be researchers primarily in academic settings. I think what we have been talking about and what we will be awarding a contract for very soon for the six month planning

process is to try to figure out if what we are trying to do is to train people to work in learning health systems, as Steve was saying, the skills and knowledge needed is likely to be somewhat different from what we have been doing to train people to work in academic settings.

What are those skills and how should the traditional training programs be different. It is not so much training to study learning health systems but rather to work in them, which inevitably will mean, of course, trying to figure out what's going on in them, and in particular, to train folks to be able to conduct PCOR within these systems.

I don't know if that is helpful in response to your question. I hope so.

CHAIRMAN NORQUIST: Okay.

MR. KRONICK: We have had some very productive talks with Joe and folks at PCORI, and would be delighted to explore collaboration.

CHAIRMAN NORQUIST: Great. Leah?

MS. HOLE-MARSHALL: I also think it is

exciting to explore this area. I was very struck by our patient panel today and one of the concepts we didn't touch on a lot but was highly recommended was encouraging how we could empower patients and how we can support through both our systems and our trainings and even our clinical training, what I would call patient empowerment or they refer to it more as patient engagement or patient training to be engaged in their own health.

So one thought I have about, I don't know if it fits into workforce training but it could, is what could PCORI uniquely contribute to others who are already doing training? So it may be that we say, you know, we want to see modules on patient empowerment/patient engagement and we will help to fund your researchers or your workforce if you have these modules present, so that we are really creating that environment where patients are equipped to be a part of the solution, and it's not just the patients that need to be trained to be equipped to be a part of it. That's all of the participants.

So I would really love to see some additional development on that concept, which may not be it known program. Can we that it in other people's programs and, you know, help contribute or pay for it if we do.

CHAIRMAN NORQUIST: Bob.

DR. ZWOLAK: The accounts that PCORI led or PCORI influenced development of new workforce programs is very exciting to me and in our application enhancement workgroup one of the hypotheses that we tested and we thought we had evidence was and in adequate number of appropriately trained workforce researchers, but to me the question is as we sort of shift from resource unlimited to potentially resource limited, how much of an investment do we have to make in this arena to be meaningful and what are we taking away from in order to do it?

CHAIRMAN NORQUIST: Barbara.

DR. McNEIL: I have a couple of comments. I totally support work in this field. One background comment I think we have to recognize is

when we train people to do work in this field, there has to be money for them to do research in this field. So we have to make sure that that's there. And right now as I look at the fellows that are coming out of residency programs, 10 years ago and Rick would probably know this as well, but they would all go into health services or research for health policy or something like that. Now the vast majority of them, at least in the Northeast, are going to work in hospitals on quality and safety and because those are hard money jobs and they don't have to worry about research. So that's just a little caveat. We don't want to train people to get all revved up about doing something and then not having any research money. But, having said that [inaudible] unknown.

Let me make two other comments. I really like the idea of AHRQ holding this planning conference. And it strikes me that there may be two levels of training for workforce that we could consider. The first is I think the one that we've been talking about here which is training

academicians -- I'm going to talk about academicians for the moment, so they actually understand how to ask questions and what are the kinds of questions that are answerable within our current framework. And that's not necessarily what comes out of the typical MPH program. It might, but it might not.

So I'm assuming that's one of the areas that AHRQ is going to go down, but the second question is -- and by the way within that there will also be some different kind of statistical skills that will have to emerge that do not come out of your average questionnaire regression analysis that's just not there.

But I don't know how many of you have tried to hire recently programmers or analysts who can merge data, who know how to deal with missing data in any way even though a statistician has told them what to do, who know how to link data -- how many have tried to hire any of them recently? And have you had an easy time? They are almost impossible to find. We've had job postings for I

don't even know how long.

So I think one of the things when we think about this workforce training is we can train all of the academicians in the world with this huge cadre of funding sources, but if there are people to help them implement their work we're cooked. So I am encouraging us to think about how we plan ahead to get this next level of people who will help implement the work that I think you're talking about Joe.

I don't know, other people may have a different view of it.

CHAIRMAN NORQUIST: Yeah, you can respond.

DR. SELBY: Sharon, I'll be very brief. Just to bag up what Barbara said in the background materials that we obtained -- mostly I think from websites, but also in conversations with RWJ. One of the reasons that the Clinical Scholars Program ultimately got defunded is because of declining application rates. Just exactly what you said. People are saying that the funding is drying up for this traditional academic research.

And the second thing is, I'm 100 percent with you, if we're going to build up PCORnet and say this is really the place where research ought to be done in the future, for goodness sakes you need a cadre of people who know how to do this. And I would say one of PCORnet's challenges is that we have a very large community of investigators involved in PCORnet with only a small number really familiar with these databases, and the capacity, and the importance, and the methods for the linkage in handling missing data. A huge issue even in PCORnet, the place this is supposed to be becoming reality.

And the last thing is I really think that in Kaiser or in elsewhere where there are people doing things such as quality, who are underprepared from a data point of view, that some of the leading candidates for this kind of training would be people who are already within their systems are supposed to be doing performance improvement quality safety work.

DR. McNEIL: I wouldn't restrict this to

PCORnet at all Joe. I think some of these issues can apply to one single -- Dartmouth or Harvard or someplace where they're just trying to look at NIH data from Proven [phonetic], Optum, or Medicaid or Medicare or Medtronics. They don't have to have sites all over the place, it's just link a couple of things together and you get the very same problems and that's actually probably more important because there are going to be many more of these one-offs then there are going to be in the PCORnet family.

CHAIRMAN NORQUIST: Okay, Sharon.

DR. LEVINE: I was basically going to make the same point Joe made. There are a lot of people currently in roles in health systems who have these assignments who are unprepared to actually fully exploit the data that they have at their fingertips or access to. And there are clinicians, you know, who every day are begging for research support to answer the questions that are coming up in clinical practice.

And so, to me investing some in folks at

the VA or a system like Kaiser or Group Health -- maybe, assuming the Group Health members like the idea, could potentially strike an oil well of interest in productivity that could be tremendously useful. There's certainly a huge receptivity to it in Joe's old division of research, physicians and clinicians are beating down the door every day demanding "Please help us answer these questions" and the folks we have in our quality and operation support function, again, just don't have the training and the skills to step up to the next level of outcomes research.

CHAIRMAN NORQUIST: So we're at the end of this time. Allen and Harlan I think if you want to make your points quickly then I think we have to wrap this up.

DR. DOUMA: Just quickly, what I'm hearing is there's a lot of demand out there from organizations out there that I presume have their overarching organization where it's even more true; hospital associations or others, and this goes back to what Leah was saying as well in patient

empowerment. There's a lot going on in the nontraditional, meaning non-academic setting that it seems like we ought to see if we can work with those organizations as well, because they are going to be very supportive of what we do and we can help collaborate with them and don't just think of your normal -- in the normal inside the box.

DR. WEISMAN: Since I'm involved with this I certainly have a lot of opinions about it, but I'm not can talk about that. I thought the comments around the table were really good and I really appreciate the board's support for the general idea and there were a lot of good comments and suggestions. But something Barbara said that struck me about the shortage of capable people trained in data analytics.

I'm involved with an organization that may be some of you know about it, it's called -- as an advisor, Drugs for Neglected Diseases and they do drug discovery, all kinds of things. They wanted to look at Big Data. There are people outside of the healthcare realm that really know how to do

this kind of programming, know how to do these things. And they are thrilled to be able to help healthcare. They don't do it for a living, they may just look at financial data on Wall Street or be involved in other types of Big Data like in the banking world, but there are legions of these people that you can so-called crowd source to help solve some of these problems. It's an off-the-wall idea, but I thought I would throw it out there because I've seen it work before and they readily come to the table and they do it after hours as volunteers.

CHAIRMAN NORQUIST: Thanks, so Joe, you got what you need? I mean, it sounds like to me there is a very enthusiastic interest in this, but we need to be very clear what the "it" is --

DR. SELBY: That's right.

CHAIRMAN NORQUIST: I think working with AHRQ on this would be very key.

DR. SELBY: Good, thanks everybody. I'm now going to ask Jason Gerson who is our associate director for CER Methods to give an update on open

science. Another topic the RTC has been looking at in depth and including a visit just at the last RTC meeting where we were urged to get some practice examples in place.

So Jason, thanks for being here and thanks for your hard work and that of your -- Jason actually has put together a team of scientists on PCORI's staff to carry this forward. Thanks to all them.

MR. GERSON: So, thank you Joe. I'm happy to be here with you this afternoon and talk about some of the progress the Open Science Working Group that PCORI has made over the last several months, so next slide please. Do I have control of it?

So this working group was convened in the summer of 2015 to basically revise the draft policy on open science that we inherited from some other folks internal PCORI and to make recommendations for how to operationalize that policy.

The working group is comprised of some -- is this coming in and out a little bit?

CHAIRMAN NORQUIST: You're good.

MR. GERSON: It's fine? Okay. Sorry. From staff members from the Science, Legal, and IT teams. They are listed for you in parens.

Today my job is to update you on some of our efforts and a apprise you of some planned activities. So, all just -- it brief road map, I'll talk about this consultation with some national experts that we've done. I'll provide a brief recap of an annual meeting plenary session regarding open science that was done in early October. I'll talk to you a little bit about a public release that we're planning for the draft open science policy, and then and with some future planned activities/action items and have it up for discussion.

So members of PCORI's Open Science Work Group have spoken with a number of leading national experts about some of the operational and technical challenges for implementing an open science policy. These conversations have been very rich and I think educational for the working group members and have focused on a number of critical considerations,

which include but not limited to operational challenges of building and maintaining data repositories; making key decisions about Centralized versus Federated IT models for data sharing; challenges that we are going to face regarding de-identification of data; the development and enforcement of data use agreement; issues of informed consent; as well as ascertaining participant perspectives on data sharing.

We held a -- many of you were attendance for this, but all just summarize. We held a very productive plenary session as part of the annual meeting. The overarching goal of this session was to discuss technical, legal, and ethical challenges to the implementation drawing on the panelists' perspectives and experience addressing those challenges. We had Dr. Francis Collins deliver a keynote to begin a session in which he spoke about the historical context of open science with a focus on some of the past and present NIH and international initiatives.

The session was moderated by Austin Frakt,

who is a health economist and has written about data sharing and transparency. Steve Goodman, who is the Vice Chair of the PCORI Methodology Committee among other roles, presented the PCORI Open Science framework and discussed some key decision points for implementation. And then the invited panelists, which included Phil Bourne, Brad Malin, and Michelle Mello spoke to a number of the substantive and technical challenges I alluded to on the previous slide.

So that was followed by very robust audience Q and A, which included questions about what data should be made available and who will have access to it. The need to differentiate between results of data, usable by general public and raw data sets that require expertise to evaluate, and some privacy and security concerns, as well as patient consent and control of future uses of data.

So some of the key takeaways from this plenary session: One is that incremental progress is worthwhile and prudent. Infrastructure which

includes the technology, governance, and staffing to support data sharing is nontrivial. The other thing that came through is, you know, PCORI is not -- need not go it alone in this endeavor. NIH is actively working in this area as well as other funders and we should seek collaborations with those organizations. And to state something obvious, that building and maintaining trust in PCORI once, you know, once we undertake this data sharing initiative it's going to be critical and that we need to find ways to ensure -- once we roll this out to ensure that data user compliance with our policies should be measured and evaluated.

So, the Open Science Working Group has drawn on the insights from our interviews as well as what some learnings from the Open Science Plenary Session and we revised the draft policy that we inherited beginning in the summer of 2015. So the key overarching goal for that policy is to articulate PCORI's commitment and vision for open science and to signal expectations for applicants, awardees, and other stakeholders. And more

specifically the purpose of the policy is to facilitate reproduction of the original analyses to decrease the integrity of PCORI-funded research findings and to promote data sharing to enable conduct of additional analyses using data from PCORI-funded studies, thereby augmenting the knowledge generated from the original study.

So just a few -- let me make a few additional points about the policy and then I'll take you through some of the key requirements for that policy. One is that in the work of the Open Science group we've tried hard to align this policy with the recommendations that emerged from the IOM report around sharing clinical trial data. So we've done that.

They had a subsection on recommendations for funders, which I'm happy to talk to talk through with you that we've tried to align the policy with. And then, we've also use the terminology -- some of that technical terminology in that report to make sure that the policy that we draft is in keeping with where the rest of research

community may be going.

This draft policy is also consistent with PCORI's funding contract, which includes an obligation to develop and maintain a data management and data sharing plan. And it appropriately creates an opportunity to add procedural details as we evolve and make decisions based on a plan pilot which I'll describe any moment.

So as you see we included the latest version of the draft policy for your review and as you've seen from that document it's organized around three sets of requirements. One is for applicants. They must demonstrate a willingness to support open science and describe planned activities that will enable data sharing in their application.

For awardees they must prepare for possible future request for data sharing by developing a data management and data sharing plan in a manner consistent with the applicable privacy, security, and other legal requirements. And just

kind of in broad strokes that data sharing requests may originate from third-party researchers and/or from PCORI program staff.

So as I signaled on the previous slide, the policy has been drafted in such a manner that will allow PCORI to incorporate additional operational details and procedures over time. In that the RTC reviewed and discussed this draft policy at they are September 2015 meeting. And the draft policy, we're planning on releasing it for public comment in early 2016 after a more formal presentation of the board for your consideration and approval. We're intending that for early 2016.

So here are some planned activities that we have in mind for the first part of 2016. So we are recognizing that these are -- kind of iterate by experience by the RTC, I believe prodded us to do so to that end we're going to work collaboratively with a handful of our awardees that are both in the general portfolio and in PCORnet, and were going to pilot some data sharing approaches. So that we begun internally to

identify some good candidates for that, they will include trials and observational studies and we hope to have those identified by the end of this month.

The Open Science Working Group is in the process of identifying a number of data repositories that already exist, these could be platforms that exist at an academic institution such as Stanford, they could be cloud-based solutions. And so, we're going to do kind of our first pass at evaluating the pros and cons of those and then kind of come up with a short list of kind of work through.

Nadine and I, as well as some others, will begin drafting draft governance protocol for reviewing and evaluating data access requests. And then, we'll have -- recognizing the depth of expertise among PCORI staff on these particular data sharing issues is not adequate or sufficient, we are going to engage a group of external experts from around country that will serve as an advisory group for our ongoing efforts. So we'll do an

initial convening of that group in mid-January and they'll help us kind of think through some of the data repository options, help us with governance, and then help us set the parameters for the pilot. And we intend to keep them engaged over the first half of 2016 to help our learning.

So, you'll see from the discussion questions that are kind of focused on the pilot exercise, they are in front of you now but I'm happy to take some broader questions or comments. So I can stop here.

CHAIRMAN NORQUIST: So let me -- I think Harlan Krumholz is on the phone now. Harlan had left. So I wanted to give Harlan since he's been very vocal about open science and opportunity to say something. Harlan are you on?

[No response.]

CHAIRMAN NORQUIST: Okay, I guess not. That's what I was told, okay. So, Rick Kuntz.

R: First of all I applaud you for doing this, I think this is really important project in PCORI in general and I would recommend two things.

One is can we make this a general model beyond PCORI? Because I think it's something that could be a real legacy for PCORI. And the two stakeholders you have to get involved is academia and industry since the vast majority of clinical trials are still industry-based. There is a lot of myths about what the problem of open science is in industry and to convene them and to walk them through the 12-step process and say it's not going to be the end of the world.

The other thing is there has to be a new method of tenure and promotion in academia. It dovetails with sharing data and that's something that's really holding academia back. So I don't know if that's overloading your schedule, but I think in the way that potentially can make this much more generalizable and kind of a big legacy contribution of PCORI.

MR. GERSON: Those are both helpful and there are things that we've been mindful of and, you know, there's obviously inherent in this work is kind of a norm changing around clinical data.

Some of this has already existed in more basic sciences, but for clinical data there's a lisp but I do think on the two fronts -- it's not overloading us, we're mindful of those and need to think about that.

CHAIRMAN NORQUIST: Bob Zwolak.

DR. ZWOLAK: It seems like such a no-brainer. I'm sure there are some real and other perceived downsides to this for those of us who are unaware could you identify very briefly identify maybe a couple different from what Rick just mentioned?

MR. GERSON: In terms of downsides, well I think part of it -- there's a couple of things. One kind of gets to what Rick was saying about the kind of incentive structure in academia so we're needing to make sure that data that's accessible and shareable still allows the initial investigator to do the work to get mileage out of it before it's shared, but that's something you hear put out there that the data is published -- people I think, historically -- traditionally, I think this is

changing but have relied on a single data set to mine for numerous publications that help them move through -- but that model's changed. That's kind of a -- you know, not to be too dismissive of it, it still needs to be contended with but I don't think it's a problem.

The other thing I think that we need to be mindful of and the people who work on privacy and worries about people hacking data, linking data. These are some of the questions around, you know, what data gets used? Is it individual level data versus kind of a more meta or aggregate level data? It's the most useful data that can be found for research purposes is the individual level data and we'll have to work hard and we're also constrained by HIPAA reality to about what, you know, what is ultimately shared and how we're going to best protect that data and we have real questions about where it will reside, kind of platform it will reside in, and for PCORI kind of thinking through the legal implications.

They are all addressable concerns and I

think my presenting to you today is a signal that we're endeavoring to meet, you know --

DR. SELBY: Just to add a third level --

MR. GERSON: Sure.

DR. SELBY: Just to add a third level to patients and researchers that Jason mentioned is we see a lot in PCORnet that you have multiple institutions that are contributing data and when you build a common data set for analyses even, you have to put in some kind of indicators of who was clustered in the same delivery system. Delivery systems are very concerned that if the data were available in some kinds of open arrangements others could get in and compare other things in their system to other systems in ways that would be harmful from a proprietary point of view.

So again, as Jason said it's not an insurmountable defect or challenge but is going to take a fair amount of thinking to surmount it before people are really ready to just sign on the dotted line to "Yes, you may have my data."

CHAIRMAN NORQUIST: Freda.

DR. LEWIS-HALL: Yeah, actually Joe I thought you were going in another direction which is there is just a flat out technical and cost barrier to this as well.

So, having the data set in an available form for sharing in addition to all of the other philosophical questions that need to be asked there are a number of logistics questions. And then who's going to pay, where is it going to be housed? Who will evaluate access to it? So I think there are a number of questions of how you actually get it done -- questions that will need to be answered as well. And one of the reasons that the pilot is helpful is it will start to, you know, the rubber is going to hit the road on these and your least know what the questions are to look at models for solving them.

CHAIRMAN NORQUIST: Harlan Weisman.

DR. WEISMAN: So I'm reacting Joe to your statement. They weren't consenting to it, but yes you may have my data. I mean, in my view anything PCORI funds belongs to the public. It doesn't

belong to the researcher. And the academic researcher -- you know, I don't think were funding things to advance people's careers. We're funding things to improve the health of the American public. I know that may be naïve, but Rick you already mentioned other fields -- physics is already open science. Many disciplines are open science. There are precedents in the medical world, The Milken Foundation are only funding researchers who are agreeing to share their data.

And so, I think I'm not saying that people shouldn't write papers or advance their careers, but they should do it in a way that these higher needs are met and I don't know whether we've explored what Milken has done and other organizations that demand this.

MR. GERSON: Yeah, I mean, that's a good point. We haven't with Milken in particular. Wellcome does something similar with they are funded work, but we'll look in Milken, too.

CHAIRMAN NORQUIST: I think the other issue here is to have this conversation about

academic enterprise so to speak, because that drives a lot -- you know, career advancement and these kinds of issues are really key. Until you change that and make a fundamental difference in how you can advance in an academic setting, it's not going to change. There is a huge issue there that --

DR. WEISMAN: So let's change it.

CHAIRMAN NORQUIST: Yes, that's easier said than done. Rick.

MR. KRONICK: The federal government, as I think folks know, you know has moved to require open science. Now from getting from that requirement to what that means on the ground, we're working at it at AHRQ. I imagine NIH still working on all the issues that have been raised or issues that we're facing as well. So I think it theory we are there, but in practice clearly not yet.

CHAIRMAN NORQUIST: I mean, as long as you get all of the parties together and agree on that and make it work, I think you can get there. I think everybody agrees that's the way to do it.

Any other comments?

[No response.]

CHAIRMAN NORQUIST: Thank you Jason very much for that.

MR. GERSON: Sure.

CHAIRMAN NORQUIST: We don't have anyone here present on the phone, so we will not be initiating our public comment period. As always, we welcome feedback at info@pcori.org or through our website at pcori.org.

Were there any final comments, questions, points? I do want to let everybody know that we had a picture tomorrow at 11 a.m. We are meeting at M Street, don't show up here tomorrow because we're not here.

DR. SELBY: We can go off the record.

CHAIRMAN NORQUIST: Okay, so I'm being told not to make any personal announcements until we get off of the record. So let me just go ahead and close by thinking those who joined us today, both in person as well as via webcast and teleconference. All materials presented today will

soon be available on our website and today's webinar was recorded and should be posted on the website by and of the week. We always welcome your feedback as I just said at info@pcori.org or through our website at pcori.org.

Thanks again for joining us and good evening.

[Whereupon, at 4:53 p.m., the meeting was adjourned.