PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

BOARD OF GOVERNORS MEETING

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B&B REPORTERS
701 Copley Lane
Silver Spring, MD 20904
[301] 384-2005

APPEARANCES:
BOARD OF GOVERNORS

Debra Barksdale, PhD, RN
Kerry Barnett, JD
Lawrence Becker
Francis Collins, MD, PhD
Allen Douma, MD [via telephone]
Christine Goertz, DC, PhD
Leah Hole-Marshall, JD
Gail Hunt
Robert Jesse, MD, PhD
Richard Kronick, PhD
Harlan Krumholz, MD
Richard E. Kuntz, MD, MSc
Sharon Levine, MD
Freda Lewis-Hall, MD [via telephone]
Steven Lipstein, MHA
Barbara McNeil, MD. PhD
Grayson Norquist, MD, MSPH [Chair]
Ellen Sigal, PhD
Harlan Weisman, MD
Robert Zwolak, MD, PhD

AGENDA

AGENDA [Continued]

P R O C E E D I N G S

[10:17 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. I want to welcome you to today's Board meeting being held in Washington, D.C., as well as via teleconference and webinar. For those unable to attend in person, instructions for logging in or calling are available at our website, pcori.org/events.

I want to remind everyone that disclosures of conflicts of interest of members of the Board of Governors are publicly available on PCORI's 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 we can

discuss how to address the issue.

If you have questions about conflict of interest disclosures or recusals relating to you or others, please contact your staff representative.

All materials presented to the Board for consideration today will be available during the webinar, and after the webinar it will be posted on our website, at pcori.org. The webinar is being recorded and the archive will be posted by the end of the week. We have two scheduled public comment periods today: one this afternoon and another just before we end. If you are interested in registering to provide public comment, please visit our event page. Alternatively, you can always email us at info@pcori.org or provide input through our website.

Finally, a reminder that we're live Tweeting today's activities on Twitter and you can join the conversation at #PCORI.

So, at this point I'll introduce Bill Silberg, who will do the roll call.

MR. SILBERG: Thanks, Gray. Debra

Barksdale?

DR. BARKSDALE: [No response.]

MR. SILBERG: Kerry Barnett?

MR. BARNETT: Here.

MR. SILBERG: Larry Becker?

MR. BECKER: Here.

MR. SILBERG: Francis Collins?

DR. COLLINS: Here.

MR. SILBERG: Allen Douma?

DR. DOUMA: Here.

MR. SILBERG: Alicia Fernandez?

DR. FERNANDEZ: [No response.]

MR. SILBERG: Christine Goertz?

DR. GOERTZ: Here.

MR. SILBERG: Leah Hole-Marshall?

MS. HOLE-MARSHALL: Here.

MR. SILBERG: Gail Hunt?

MS. HUNT: Here.

MR. SILBERG: Robert Jesse?

DR. JESSE: Here.

MR. SILBERG: Richard Kronick?

DR. KRONICK: Here.

MR. SILBERG: Richard Kuntz?

DR. KUNTZ: Here.

MR. SILBERG: Harlan Krumholz?

DR. KRUMHOLZ: Here.

MR. SILBERG: Sharon Levine?

DR. LEVINE: Here.

MR. SILBERG: Sorry. Freda Lewis-Hall?

DR. LEWIS-HALL: Here.

MR. SILBERG: Steve Lipstein?

MR. LIPSTEIN: Here.

MR. SILBERG: Barbara McNeil?

DR. McNEIL: Here.

MR. SILBERG: Gray Norquist?

CHAIRMAN NORQUIST: Here.

MR. SILBERG: Ellen Sigal?

DR. SIGAL: [No response.]

CHAIRMAN NORQUIST: She's here.

MR. SILBERG: Yeah, I've seen her. Harlan Weisman?

DR. WEISMAN: Here.

MR. SILBERG: And Robert Zwolak?

DR. ZWOLAK: Here.

MR. SILBERG: You have a quorum.

CHAIRMAN NORQUIST: Thanks, it would have been interesting for people in the room to not say they're here. I don't know what that would have meant?

So, okay, all right. In context. Yeah, as a psychiatrist, I mean, I can understand. I'm not sure I'm here, either. But, anyway, okay.

Joe, I'm interested in Joe Selby and how he's in the room. Do you want to make a brief comment?

DR. SELBY: No, I want to make some lengthy comments.

CHAIRMAN NORQUIST: That comes after this, I know.

DR. SELBY: Good morning, everyone.

CHAIRMAN NORQUIST: Is that it?

DR. SELBY: That's it.

CHAIRMAN NORQUIST: That's it? Oh, oh, really brief. Okay. I'm shocked. I've never heard you do it that way.

[Laughter.]

CHAIRMAN NORQUIST: Okay, so now the first item, actually, is the approval of the minutes from our April 21st Board meeting which actually, Kerry, you were at that one, so I need a motion to approve the minutes?

MR. LIPSTEIN: Actually, Gray, I chaired that one.

CHAIRMAN NORQUIST: You chaired that one? Oh, you got stuck with it at the end.

MR. LIPSTEIN: And it was especially efficient. I just wanted you to know that.

CHAIRMAN NORQUIST: Oh, excellent. Okay, so I need a motion to approve the minutes.

DR. ZWOLAK: Moved.

CHAIRMAN NORQUIST: Okay, thanks, Bob. And then a second?

MS. HUNT: Second.

CHAIRMAN NORQUIST: Thank you, Gail. Okay, is there any discussion about the minutes? Any corrections of Steve's wonderful job?

Okay, not hearing any, I think we can just have a general approval. Everybody in favor?

[Chorus of ayes.]

CHAIRMAN NORQUIST: Anybody opposed and anybody abstaining?

[No response.]

CHAIRMAN NORQUIST: Okay, so that takes care of the minutes. And then now, Joe, you get to have your lengthy comment.

DR. SELBY: Thank you, Gray. And it's a pleasure to be here with all of you again. I'm going to start with some brief but important items on a number of topics that we won't get to talk about -- most of which you won't talk about otherwise today.

The first is real exciting news about the chair of our Methodology Committee, Dr. Robin Newhouse, who is leaving the University of Maryland to become the Dean of a three campus school of nursing at Indiana University. Indiana University is very excited and my understanding is that Robin is very excited, as well.

And we're very excited because -- now watch the screen -- she was the chair of the PCORI

Methodology Committee and she remains the chair of the PCORI Methodology Committee. And the chancellor who hired her, I presume, said that it was a real honor that she chaired the Methodology Committee of PCORI, so we're really grateful that he or she understands that and that Robin wants to continue and is able to continue.

MR. LIPSTEIN: Joe, why does the before picture and the after picture look identical?

DR. SELBY: It's perfect plastic surgery. I mean, it's wonderful.

MR. LIPSTEIN: It's a quick transition.

DR. SELBY: Okay, so just, again, congratulations from all of us, Robin. It's something.

[Applause.]

DR. SELBY: As Steve Goodman pointed out, chairs of the Methodology Committee, the next step tends to become a Dean, because it's happened to both of our chairs.

Okay, this is in the vein of beginning the announcements about activities we do to

disseminate. That is an activity that will be big in 2015 and beyond, and this is dissemination through one of the many vehicles that are sometimes useful and that is continuing education, continuing medical education. We have contracted with a pair of institutions, Prime Education and Baylor College of Medicine, who are very proficient in this work. And we are designing continuing medical education activities directed towards physicians, physician's assistants, nurses, nurse practitioners, pharmacists, health professionals, and we're starting with two topics.

One, the methodology standards. So we actually are doing several activities now to formally disseminate the methodology standards to professionals and to professionals in training. And we are also disseminating the results of the very first published comparative effectiveness study funded by PCORI, a study of intravenous versus oral antibiotics for children when discharged from a hospital after severe infectious osteomyelitis.

The CER study showed really quite clearly that oral antibiotics were every bit as good as what is called a PIC line, an intravenous line for delivering antibiotics, with none of the complications, none of the infections, the clottings, the breaking off of catheters. So an editorial that accompanied it said this is likely to change practice, which, as you know, is just what we're shooting for.

So that will be disseminated by -- we're preparing a video that involves some of the scientists that were involved in the work, so that's a piece of news.

You recently approved our revised peer review and public release of research findings, policy, and process after a period of public comment and we are now finalizing technical and other issues to implement this process, including we have an RFP that is out and will be awarded for the management of peer reviews. So we will contract for the management of peer review with a professional organization that does that. We

anticipate making an award in June.

We are about to release an RFP for a contractor to manage development of the lay summaries that will be posted on the PCORI website. I have a plan in process to modify the contracts that are already in existence -- and that's 200 contracts, roughly, 200 plus -- to include more details on what we are requiring, in terms to prepare for peer review and public release.

All of our future PFAs and award contracts will include the new, detailed peer review language from the outset. And the first funded projects to be peer reviewed, and results released, by the end of this calendar year.

More new news, developing news, breaking news on our PCORI first annual meeting. The dates are set, October 6th to 8th in suburban Washington, D.C. The goals of this are to report on our accomplishments to date and update the research community and the nation on our plans for the future, to highlight contributions we have made. We'll be marking our fifth anniversary. The

audience will include awardees, perspective awardees, researchers, as well as stakeholders. Also including the broader stakeholder community that's interested in PCOR. And we anticipate about 500 to 700 people.

There will be a joint session -- the first half-day is a joint session with AHRQ, so we're following right on the heels of AHRQ's annual meeting, and AHRQ is working closely with Academy Health in planning and in delivering this meeting. There will be an open plenary with a stakeholder panel on the state of PCOR; a plenary on patient engagement and what we've learned about it and where we think that's headed, its impact; and then breakout sessions centered on clusters of our pilot projects that fit various themes, other clusters among our funded research -- our primary comparative effectiveness research, as well; themes like shared decision-making or community health workers or patient navigators, just to name three possibilities.

There will be learning labs focused on

skill-building and information sharing. And there will be a summit of organized groups of researchers, so organized by major initiatives to coordinate around aspects of methods or around aspects of engagement. So that's the first annual meeting. I hope you will be able to make it.

And now I just want to put up for the first time at a Board meeting the 10 pragmatic clinical studies that we funded. We are intensely proud of these studies. I think you will take a look at them and see that they are the kind of studies you've been talking about. These are large numbers -- 10- to $15 million studies, for the most part. They are head-to-head comparisons; they all are, although some are head-to-head versus carefully defined usual care. But this first one, breast cancer screening that is tailored to the individual's risk and preferences versus just annual recommendations for annual mammography, a really exciting study that kind of gets behind the recent U.S. Preventative Services Taskforce announcement and says, what would happen if women

did follow that approach?

The next one is annual versus biannual CT scanning. After-patients have been found to have small, potentially cancerous nodules, but probably not cancerous on initial screening CT scans. And I won't go through all of these, but here's the first five, and then here are the next five. So you see a number of them involve medications or anesthetic procedures: nerve blocking versus general anesthesia, proton beam versus photon beam radiation therapy. So, 10 studies that we are intensely excited about and looking forward to getting started and managing, and we hope that there are many more. We anticipate that there will be many more.

Coming down the pike, a product of our advisory panel and SOC deliberations, our due diligence of staff, but also a product of the research communities responding to our high priority list of topics. So there's a lot of thinking from the research community built into these, as well.

[Sound dropped.]

DR. SELBY: -- cycle's worth. You may recall that we announced that we would fund 6 to 9 per cycle, so that is 12 to 18. We have 10. That is of some concern to us and we want to initiate a discussion with you.

I want to show you first some of the evaluation activities that we are already undertaking to try to understand why, instead of getting 18 applications after 2 rounds, we only felt we could fund 10. We get a lot of applications. We remove a number that are not responsive and don't meet priority criteria at the level of the letter of intent. But between that and then the merit review, we have not come up with six to nine per cycle yet. So what we're doing is intensively and actively reaching out to the research community to increase awareness and increase the number of LOIs and applications.

So we think that there is still work to do with organizations like clinical specialty societies -- the clinical research community, in

general -- about these awards, as well as the primary care research community. We've been talking more intensely to the primary care research community about taking the lead on some of these.

We're working with stakeholder organizations to increase the number of high priority topics. We have now about 22 topics and 2 or 3 of them may be coming off the list in the not too distant future because we funded work. So we need to keep the pipeline open to get more high priority topics onto this list. We're working hard to refine the PFA to be even clearer on the requirements and expectations for these awards, so we're trying to be crystal clear what we will be attracted to funding.

We're looking at merit review intensively and in several ways, working with our merit review teams to assure that the reviews really capture PCORI's review criteria as well as humanly possible. We are considering the notion of increasing the proportion of LOIs that get let through to fund full proposals. In one way we felt

it was a favor to researchers to say this doesn't stand much of a chance. We'd recommend you not spend your time writing 25 pages.

On the other hand, we're now just letting approximately 25 to 30 percent through and there may be some room to let a somewhat larger number through, given that the LOIs are really just 4 pages. And by sending back comments and concerns after the LOI, some good proposals may get developed in response.

We also -- it needs to be mentioned that we haven't had a cycle yet where resubmissions have come through. So resubmissions, in the broad announcements, as you'll recall, get a higher acceptance rate. So that may drive up the number of awards in future cycles as we receive resubmissions.

We are evaluating research readiness. And you're going to hear a bit of data on this from Lori Frank later on this morning about that. Just what are researchers thinking about organizing and leading pragmatic studies? Pragmatic studies are

different.

And we're, lastly, surveying researchers who have recently published good CER. And you see a lot of good comparative clinical effectiveeness research being published in major journals. We're going to survey some of those researchers to see if they're aware of and have thought about using PCORI as a funding source.

So those are about 10 activities that we've undertaken, but your thoughts in a minute on what else we might do would be very useful.

So here's today's agenda. After we conclude this -- and this is the last slide -- we're going to move right into the Dashboard that covers the first two quarters of 2015. And you will see that we've made some changes in response to your comments after the last Dashboard was reviewed, and we will anticipate that you'll have more suggestions for changes this time and we will incorporate those.

Then you'll hear a financial update. Just five months of financial data are available at this

point, but it's enough to show you some trends that Regina Yan will explain in more detail, along with Larry Becker from the FAC.

We also have a charter to present to you for a Scientific Publications Committee. And Bill Silberg will explain that we have been working with a Scientific Publications Subcommittee, but we find several reasons to elevate that to a committee and putting that to the Board for approval.

We have three demonstration projects for PCORnet that have been recommended by one or two strategy committees of Board for approval today by the full Board. And we will hear from Robin Newhouse with an update on a range of exciting activities the Methodology Committee has underway. Lori Frank will present what's going to become a regular feature, I think, of Board meetings: our data.

About a year ago, Harlan Krumholz charged us with getting a better handle on our data, and we've been working hard on that. And I think the news for you all is that we have a lot of data now

on our applications, on our processes for monitoring them over time, on surveys that we've conducted externally, and we're looking forward to having more and more meaningful discussions from these data about the impact of PCORI.

You will hear an update and an overview from Jean Slutsky on PCORI's Communication and Dissemination Research Program, so this is not dissemination activities, this is research on dissemination. As you'll recall, Jean wears two hats and one of them is as Program Director for this research program.

And then you will hear from Christine Goertz, Chair of the SOC Strategy Committee on the operationalization of how we do topic prioritization and how we make it transparent. So that's the agenda for today.

And I just wanted to briefly put a couple of questions to you. One is, do you have other thoughts, suggestions, questions about increasing the number of high quality Pragmatic Clinical Study applications and subsequent awards?

Any comments or questions, also, on the annual meeting?

CHAIRMAN NORQUIST: Okay, so why don't we take them one at a time. Which one -- why don't we go with the annual meeting first and then come back?

DR. SELBY: All right.

CHAIRMAN NORQUIST: Are there any comments about the annual meeting? What are the dates again?

DR. SELBY: October 6 to 8.

CHAIRMAN NORQUIST: Here in Washington.

DR. SELBY: Or Virginia?

CHAIRMAN NORQUIST: Crystal City. Okay, yeah, so technically Virginia. Yeah, Harlan?

DR. KRUMHOLZ: I just wonder what would, for you, represent a successful meeting? What are your key metrics for saying that we've achieved what we wanted to achieve with the annual meeting?

DR. SELBY: Well, off the cuff a bit, I think that continuing to build the PCOR community, bringing researchers and stakeholders together in

ways that go beyond where we've gotten to this date; seeing our awardees in person; seeing the results of bringing a number of awardees around a particular research theme together. Those are some of the -- if we accomplish those, we'll consider it a success.

I am probably skipping over a couple. I'd welcome your thoughts.

DR. KRUMHOLZ: Yeah, I just wonder whether or not it will be good for the annual meeting to be directional in the sense of one of our goals is to integrate this model of including patients in with the research application. And so that what we were trying to do was show successful case studies of places where the patients made considerable contributions, even in process of this, short of having finished it, but even in the development of the application and demonstrating that it wasn't unnecessarily burdensome, that it did add to the perspective of the researchers. And these would just be stories.

And you can imagine assembling funders who

could be around and talking about if this model works, how would you think about maybe this working in RWJ, NIH? AHRQ, of course, is going to be there. And you get groups and panels talking about if this is a new wave, how will it best get someone like John Wilbanks, who's coming from, you know, Commons and talking about consent, and talk about issues that lawyer Ellen's been working with groups already and how she's been able to funnel in the patient perspective for this. So it's not just -- it's both from up and down that we do this, so it's directional.

We bring in people who are organizing large national scientific sessions in my field, like the ACC and AHA, who start saying, what would it take for you to start incorporating patients into your national meetings in ways that would get people to start thinking about the patient perspective in the interpretation of research results or in the formulation of the next generation of research questions? So that that way, we're not just using it as a way to display

and say, hey, look, we're the great PCORI and look at all the stuff we've done, but that there's a directional goal to what we're trying to achieve.

And one way that we measure our success is, have we moved our agenda in terms of what we've now internalized? And we begin to socialize that thinking and we develop the stories and evidence behind the idea. That's work that Mike Lauer had done with regard to how did it affect peer review?

We have different streams where we've said incorporating patients into peer review, what did it mean? Incorporating patients into research proposals, what did it mean? Incorporating patients into scientific presentations, what did it mean? And we begin to get a whole theme of momentum around patients at the center. And the world didn't fall down.

A lot of people have said this, well, we got off track because we're talking so much about the patient. Well, we thought that was important and, in fact, we felt it's an essential peak for the future, not a sideshow. But, anyway, it's just

an idea of where we could go.

CHAIRMAN NORQUIST: Any other comments about that? Oh, I'm sorry, Bob?

DR. ZWOLAK: So this is very exciting. When I think about the five-year anniversary, though, I suspect that the world will anticipate that we have a fair number of research results. And so I'm wondering how many of the pilot projects or other projects will have been completed to that they could be showcased at this meeting.

And also two of the big changes that you've described for PCORI are the new peer review program and the contract for that, and production of the lay reports, the lay summaries of the research. And the contracts will be rewarded, I guess, this summer. Will that process be spotlighted? Will any of those reviews be done? Will there be a summary of how that new system is working and what the impacts are on the research, on the scientists, and on our patients?

DR. SELBY: I'll take that as a suggestion back to the group and I suspect that, indeed, it

will be. In fact, Orlando is nodding in the back, so, yes. Thanks.

CHAIRMAN NORQUIST: But the first question he asked you about?

DR. SELBY: The majority of pilot projects will indeed be done by that time and, yes, they're definitely going to be presentation of results. No doubt.

CHAIRMAN NORQUIST: Gail?

MS. HUNT: Gail Hunt, Board. It strikes me again, to follow up on what was said about, actually, this is the meeting that Gene Washington always talked about, saying that we needed to have a large meeting that really would put PCORI on the map for comparative effectiveness research. So I think that's what we should kind of keep in mind, that this is going to be the opportunity for the whole CER community, but not just them, but Congress and other people that are interested in how well PCORI has done. So we've got to be able to showcase what we've done so far, what's actually been implemented, if we have anything that's been

implemented. So I think that that's really what this PCORI meeting should be about.

CHAIRMAN NORQUIST: Thanks. Yeah, I guess it's not only just the results, but what's in development also, too, which is key. Because that's some of the questions that we get when we go around and talk to others.

And I think, Gail -- I thought you might also -- this is something that I've talked to Joe and them about, about bringing other stakeholders there even for presentations, you know, about their interest in this and that kind of thing, and not just our individual investigators, and stuff there. So that's another thing that we should do. But, please, if you have other suggestions long before October, please get those into Joe and his staff.

Okay, so let's go back to the first one because that may be a little more conversation about suggestions for increasing the number of high quality pragmatic clinical studies. And I'm going to go this time this way. Francis is up next and won't come last.

Francis, you're up first this time.

DR. COLLINS: So I think it's really important to have this conversation because I do think these large-scale pragmatic clinical trials are a major contribution PCORI can make, where you are putting sufficient funds into a really important question and making sure the study is designed well, to give answers that can be almost immediately relevant for clinical practice. So one does have to wonder, why is the world not beating a path to the door with fantastic applications, considering there's a fair amount of money being made available here?

And I guess part of the question here is whether we just have a workforce problem where there are not that many experienced investigators who are, in fact, able to mount a credible proposal that passes through your appropriately rigorous peer review process, beginning with a letter of intent and then going on to the full thing. And if that's the case, what do we do about that?

I guess part of the question also has to

come back to how will the landscape change with the advent of PCORnet, which presumably ought to provide additional institutional capabilities for studies of this sort and maybe could be partly a solution to what is currently, apparently, a bit of deficit in capabilities out there?

I'd just be curious, from the way in which this has gone so far, whether you have a thought about that? Whether this is, in fact, a workforce problem and, if so, is PCORnet going to help?

DR. SELBY: Excellent question. I think we do suspect that that is part of it, particularly changing from a standard research platform and approach to a pragmatic approach, so a lot of the people that asked the really interesting clinical questions have not ever really done it in concert with delivery system leaders before. So, yes, I think we're going to have just be encouraging and supportive and push people to think about doing it that way.

CHAIRMAN NORQUIST: But do we have any data, I mean, to inform? And do we have any

qualitative data where we've gone to the investors -- because we all have individual reports. Like I've had some that said, well, there's not enough money. I mean, it's a lot of money, but some people want even more for a bigger kind of trial.

DR. SELBY: I actually think that when Lori makes her presentation -- we conducted a survey of CER researchers near the end of 2013 -- is that right, when the survey actually went out? 2014, okay, good. So it's even more current than I thought. That does have some hints that that may be part of the issue.

CHAIRMAN NORQUIST: Yeah, so that's not surprising. Let me just ask you a question on the survey. Was that conducted when the pragmatic trials were out there for people to come in? So some of these people would have decided not to or would have been thwarted, if you will, at the LOI phase or something. So we'll get some feedback from them, as well, right? Okay. Ellen?

DR. SIGAL: So this is complex. I agree with Francis. We took on -- I work with Steve

Clauser in the cancer world and work with professional society patient groups, NCI, and many others, other stakeholders, to really see if we could come up with the right pragmatic trials, and what we got back can be very frustrating.

Part of it is fitting our criteria, part of it was understanding what exactly we want that's different from what they were doing, and the other is the amount of time we need to get the answers. And the answers that would be relevant in three to five years is also a problem, but there are a few that are coming in through the pipeline that I think may be interesting. But when you really drill down to what we're looking for and what the investigators had or what other groups had it didn't really fit. It was really kind of surprising and, frankly, disappointing.

DR. SELBY: It's hard work.

CHAIRMAN NORQUIST: Is that you, Bob, with your card? I can't tell if it's you. If that's not Larry's, it's your card up, Bob.

DR. JESSE: I'm very proud of what PCORI

has done. I think we've made enormous accomplishments, but leaving research potentially allocatable money on the table, I think may be our biggest problem that we have to focus on right now. And there are some data here -- there are some slides upcoming which are pretty interesting, but I think potentially focusing even more on the people who's applications were rejected and, also, potentially on those who were accepted.

It would be interesting, that in itself might be a research study, the feedback from acceptance versus rejected people. But I think we need to really focus on this issue and figure out how to optimize our methods to get the right research chosen and funded because we have, as I look at this budget material coming up for the next six or eight months, we have just an enormous amount of money on the table that we can allocate. And I think it's our obligation to do it effectively and get it to the right people and get it out there.

CHAIRMAN NORQUIST: Yeah, but I think you

would also agree that we want to do it for good things.

DR. JESSE: Right, yeah, high quality.

CHAIRMAN NORQUIST: I think one thing that we may want to think about also is the mechanism, which one of the problems is relying on investigators to come in with their individual ideas is sometimes a problem. I learned that when I was at NIMH and we did it. We had to go out with specific contracts, asking people to come for a very specific -- and we may need to think that that's what we're going to have to do to push the field to do it. And we could take some of this money and say, this is really the key topics we want to come together as a group and, actually, we got more that way than in waiting on some investigators, kind of sitting around saying, hey, I think we go -- well, we'll wait a year or so. Do you know what I mean?

Okay, Christine?

DR. GOERTZ: I just echo Bob's concerns, but also to agree completely with Gray that we want

to be funding more research, we want to make sure that that research is of a high quality. This is something that the SOC has been talking about and we're starting to have some conversations about, are there some creative or innovative things that we can do in regard to, for instance, looking at applications that may not have scored as well as some other applications, but for which the ideas or concepts are particularly intriguing? Are there some ways that we can work with investigators to bring the research forward in a more streamlined pathway than just the normal continued resubmission process?

And, also, looking at how can we -- when we talk later about the research prioritization process, we're going to be talking about how almost a year ago you voted on our topic prioritization process and we've really spent the last year streamlining that, operationalizing it, testing it out, and realizing we actually need to make a couple of changes, but to make that more transparent and operationalize it so we're able to

move forward and so that we are able to put -- to make more clear -- to have more targeted funding announcements out there and to be more clear about the pragmatic trials that we're most interested in.

CHAIRMAN NORQUIST: Thanks. Rick?

MR. KRONICK: One of the items on the list that Joe had was around expanding the list of prioritized topics, and we may want to defer this until this afternoon's conversation when we talk about the topic prioritization process. But a different approach and one that's been discussed in the SOC is not putting as much emphasis on list of prioritized topics. And to say to the field, here's the list of topics, but we're actually interested in any topic that meets the following criteria.

And I'm sure that we could, and we already have, come up with a set of criteria, but we could emphasize that set of criteria about treatment A versus treatment B, topics that clearly are important to patients and their outcomes, et cetera, et cetera, and could not put as much

emphasis as we do in the announcement on the list of prioritized topics. I doubt that's going to fully solve the problem, but it might increase the number of high quality applications coming through.

DR. McNEIL: Harlan?

DR. KRUMHOLZ: So everyone's heard this before, but I'm just going to raise it again. I do think that a novel, innovative approach is to ask people to make proposals about questions without having to include in the application execution of the study, and that the PCORI goes out for contracts to execute the studies separately from the application to fund the study. And so that someone has to come in with a ballpark about what they think it's going to take to execute it. We think this could be executed for X-amount of dollars, but that the funding is ordered to provide the scientific oversight, and includes patients and investigators together to propose questions that they think are important, that they would like to be able to run the studies for.

I think in this country there are many

people who don't have experience running efficient trials and so that restricts it to a small group of people who know how to do that. On the other hand, if you said to PCORI, we are looking for proposals -- and that's going to have everything in the proposal except the part about how it's going to be executed and then we're going to go out to Quintile, DCRI, all the major vendors, and say, are you interested in bidding on this trial? And here's the range of money we've got available to execute this study. And I'm just going to say again, to me that just seems like the most efficient way to conduct research because the execution of the actual study is a commodity and people should be able to bid on it an do it efficiently.

The scientific part of it is what -- it takes a different skill set and providing that oversight, and industry does this all the time. Industry sits down and says, here's our question. They put together the entire protocol and then they decide who's going to execute this for us. And I

just don't know why we wouldn't want to try that model. To me it empowers patients, also, because it says that you don't have to get in the weeds here about exactly all these issues that are bedeviling and difficult in terms of the execution side. Now, they're going to have to work closely, and they would be part of the PCORI team in selecting the vendor, but anyway, I think this could be a way to do this.

The other thing is endorsing Gray's ideas, which I think this is prime for qualitative research to understand. It's market research, really. Why aren't we exciting more applications? But I think that's a separate issue. So it's up to us to innovate this idea and figure out how we can lower the bar for people getting involved as principle investigators.

CHAIRMAN NORQUIST: Steve?

MR. LIPSTEIN: Gray, in answer to your question, and in response to Francis' comment, we may not have data, but we have a history. So, Francis pointed out that we set national

priorities, we create research agenda, we put together a pool of money and the research community didn't necessarily beat a path to our door. And as Bob pointed out -- Bob Zwolak pointed out, we at PCORI have never been able to move our budgeted dollars out the door as fast as we have wanted to.

And so, I put those two facts together and I come up with an observation that we've always had to ramp up from one cycle to the next. We've never been able to start out on our first cycle and move out all of the money we wanted to move out in the timeframe we've wanted to move it out. And so part of it is -- maybe the observation is that the research community is an adaptive group and that behavior modification of the research community doesn't happen quickly or easily. And so, just with our first round of pragmatic trials, maybe we're in the ramp-up phase and the research community is adapting to our priorities, or methodology standards, and that with each successive cycle we will see increased participation.

I would like to encourage us to be patient and not necessarily to accelerate the funds flow out the door if it would mean any compromise of our scientific rigor or our priorities. What we've learned in all of our previous cycles is that either through the resubmission process or through the passage of time, the research community does respond, but it doesn't happen quickly and I don't know that we should expect it to happen quickly this time.

If this were the second cycle or the third cycle and we still had an absence of interest or participation, that would be much more worrisome. But I don't know that we should overreact necessarily. But I do think we have a history here, so we ought to look back in time and say, what is the normal ramp-up of participation in either our broad or pragmatic or focused studies, and shouldn't we anticipate that the second and third cycles will be more successful, if you will, than the first?

Joe, you probably can comment on that.

DR. SELBY: Well, you know, we've done two cycles already and we have actually four cycles in terms of the number of times people have submitted. And, actually, the number of applications is going down, not up.

Good, I think we should --

CHAIRMAN NORQUIST: Harlan has one last comment, if he'll be quick and --

DR. WEISMAN: I wanted to add to the Harlan Weisman chorus and maybe elaborate. And I thought, Harlan, in the past you've even suggested this, which is intramural research. We have topics, we have priorities, and while I agree with your suggestion, why not take some of the topics -- if we're not getting outside people to do them and we think they're important areas, what's preventing us from doing that? We can hire those CROs, we can oversee that research and we can conduct that research if we agree to our prioritization process that it's important. And if we're not getting high quality in extramurally, let's do it ourselves.

CHAIRMAN NORQUIST: That's what we did

when I was at NIMH, we did it that way. We came up with the topics and we did it. And then the other thing is, quite honestly, we asked investigators who contracted with CROs to do it, they didn't try to do it themselves.

DR. WEISMAN: Is there a reason why we're not doing that?

DR. SELBY: No, I think we have heard Harlan's suggestion before. It is a real deviation from the way that we made money available in the past, but I'm increasingly persuaded that -- and probably on the SOC would be a good place to discuss that.

I mean, in some ways our targeted announcements go a little bit in that direction. They say, here's exactly what we want you to study. But we could go a lot further and we could go to the point of contracting with the -- selecting someone.

CHAIRMAN NORQUIST: It could still be peer review. You put the topic out to them and they can still be peer reviewed. That's what we did. We

had competitors, but it was still peer review, so I think there's an opportunity here.

DR. SELBY: Okay, thanks.

CHAIRMAN NORQUIST: So we need move on now to the Dashboard report?

DR. SELBY: That's right.

So this is Quarter 2 of fiscal year 2015 and I'm going to point out some noteworthy items, and one thing you'll see is there's more yellow on the Dashboard. There's no red, which is a long time, and I don't want to pass this slide without giving my friend Winston Churchill his due. And that's what we do with this.

I also don't want to go any further without thanking Michele Orza and Katie Rader, and Lori and the Evaluation Analysis team, and really every program director and chief for their contributions to this Dashboard and, actually, for developing Dashboards in their own sectors, as well.

Questions for you are, do you see any need for further action? We'll talk about some actions

that we're taking. Do you see a need for further action and response to any of the indicators discussed today? And, as always, what would be better? What would be a better indicator? What would you like to see that you're not seeing that conveys the status of our work?

So here is our second quarter Dashboard. You can see, first of all, that it's always now continuous over time, with multiple quarters so you see trends. Starting in -- and I'm going to mostly focus on the yellows, but I will call out a couple of green ones, as well.

In the funds committed to research, you see that we pretty much hit our mark in Quarter 1, fell behind in Quarter 2. My understanding is that's basically the result of not filling out -- not completing MOU for a large amount of money, with NIH, I think, that we had originally anticipated would be completed in this quarter.

A little bit, I think, is also due to shortfall in the first cycle, but not the second, the first cycle of the pragmatic clinical studies.

As things fell out, there wasn't a lot of commitment in the first two quarters to begin with and there will be more in the third and fourth quarters, including today.

The completion of projects is really minimally behind, really. And, in fact, we're only at Quarter 2, so you see. I think we fell two to three projects short of being completed on time. These are pilot studies. This means the final report is in and I think these two or three, actually, it's my understanding is they're very close.

Regina's going to go and -- moving down this first column of yellows, the expenditures, you'll see that not only is the research budget pretty much on track and we actually count as spent that MOU money, and that's the full explanation for the shortfall in Quarter 2 of the research actual, compared to the research budget. So, other than that, our research spending is just what we projected.

In terms of the other budget, you'll see

that we're well under on spending for things like staff and other expenses. And Regina in her fiscal update will give you the explanations there. I'm going to just skip over projects awarded and, basically, we're awarding; you are party to and aware of everything we award.

Journal articles published, they tend to accrue sometimes in arrears, so that downward slope in journal articles published by awardees is likely to be corrected as additional time goes by. This second quarter just ended and we'll find others, but the news is that we're beginning to see numbers of articles. Even just in this last quarter 10 articles were published from PCORI-funded projects.

The percent of projects meeting all milestones -- upper right-hand corner -- if you look at recruitment, recruitment is meeting milestones. I think I have a little bit more data on that in an upcoming slide, so I won't say any more about that nor about the BP earnings and CD earnings. Those are kind of these curious metrics, are you meeting all of your milestones? And as

time goes by, you have more and more milestones to meet and you, therefore, can be more likely to miss at least one. So I don't think -- we won't have those on much longer.

Uptake of methodology standards, we'll talk a bit more about that in a minute, but also to say that two big events are happening this quarter that we think will hopefully create a bump-up. Part of it is just saturation. That year after they're out, most people who've commented on them or looked for them have done so. But we are -- two major efforts are underway now to further disseminate the methodology standards.

And then on PCORnet, this is always hard for me to look at, but a little white box with a checkmark means it was done on time in the right quarter. The purple is where we are right now. And where we are right now is we did not get the governance policies approved in the second quarter as hoped. Rachael told you that we got more than 400 comments. Those comments are being processed and I think the governance policies are likely to

be approved in the third quarter.

In the third quarter you see the aspirin trial will be awarded in the third quarter, and we hope that that's a little bit later on this morning.

And we always include one story, one qualitative story. This one comes from the University of Pittsburgh, where they established in 2011 a patient-centered CER unit at UPMC and wanted to develop an infrastructure for conducting PCOR, or Patient-Centered CER, to promote collaboration on CER across the university and to take advantage of new funding that is PCORI.

You look at the aims there, but we hear stories about this from a number of academic institutions, that they have set up particular seminars or established groups to discuss and monitor PCORI and to discuss the methods around Patient-Centered CER. So, quite nice, quite exciting, always glad to hear about events like that. Here's some of their activities: Establishing the HIPAA compliant data center,

establishing training and educational opportunities, and putting an emphasis on stakeholder engagement. Also encouraging people to be PCORI reviewers, thank you very much UPMC.

Okay, so the milestones again, meeting the recruitment for milestones, we talked with science leadership on Friday and part of this seems to be that we don't really have the greatest of metrics yet. We're separating out what part of recruitment shortfalls were due to a failure to get started on time versus a failure to recruit once started.

IRB approval seems to be not slowing things down that much, so 76 percent of -- or, no, these are numbers, these are not percents. Of percents, well over 80 percent are getting their IRB milestones done on time. You will see that there's a small number of projects -- and I'm not quite sure what those are. Those are denominators, and a very small percentage of those are payment holds for programmatic reasons. So we do withhold payment from time to time, and we also modify contracts from time to time, to meet shortfalls.

It could eventuate at some point in the decision to terminate a study early, if recusal, for example, is just not happening. It's just not ethical to continue the study. We don't have any instances of that to date.

These are -- I mentioned that there are 11 studies and here are some. You'll see most of them look like they come from pilot projects or they're preliminary reports from CER studies. There's the one study that I mentioned already, the comparative effectiveness research study of the intravenous versus oral antibiotics in acute osteomyelitis. But you see a lot of these are setting the stage for the CER findings that will come.

You can find, and the public can find, at any point, a description and a link to the abstract of the study, for any study that's had a publication. And it's called PCORI in the literature and it's right on our PCORI website.

So, next quarter you're going to see some -- we're planning to show to you for the first time some new metrics on early dissemination and update,

the average impact factor of our published studies, and the percent of articles that show up in top-tiered journals.

And then in fourth quarter, the citations of these articles. So they've been out now, some of them for a year. How many times are they being cited? We'll also look at some alternative metrics, like media coverage and uptake into systematic reviews or guidelines. So these are coming. It takes a little while, first of all, for it to happen, and then for us to capture it.

So I will close that and ask if there are any questions, needs for further action, if you saw anything that troubled you. And also suggestions for further improvements, new metrics for the Dashboard.

CHAIRMAN NORQUIST: So, Rick, it looks like you're up.

MR. KUNTZ: Yeah, thanks, Joe. This is exciting and I would just suggest that we kind of drill down the publications a little bit more. And I think we probably have a fiduciary responsibility

to make sure that the negative and positive results are published in a timely fashion.

So it would be interesting to be able to follow when the sites were finished, what the project is, what the metrics are on that. Break it down by original results versus editorials, and also peer review journals versus not peer review journals. But properly get four or five pages for us on that because I think it's ultimately the metrics that establishes our success and is going to be on the publication side.

CHAIRMAN NORQUIST: Thank you. Larry?

MR. BECKER: So we're about to build a wave, hopefully, of new projects completing. What do you think our greatest opportunities are to make sure that that flows smoothly out and we can handle the volume that we might anticipate coming?

DR. SELBY: Well, the first thing I'll mention is our peer review and public release of findings process. And this is in answer, in part, to Rick, as well. And we're committed to posting lay abstract, technical abstract on our websites,

as well as seeing that the data table gets posted on clinicaltrials.gov, and a full, final report within a year of the completion of the work on our website. So I think there are some ways in which we are trying to assure the smooth flow outward.

Also, though, I think our whole dissemination program will gear up as the number of publications and completed projects takes hold. Using the dissemination plan, that really pointed to a lot of different tools. One of the critical things we need to do is develop the pathways that say, here's a particular finding, what's the next step? Does this need additional research? Does this need to be tossed in to a systematic review or a meta-analysis, even? Is it ready, really, for dissemination and implementation? If so, given the findings, what are the right mechanisms?

So I think you will be hearing from Jean later about the evolution of this sort of flow chart for dissemination.

CHAIRMAN NORQUIST: Francis?

DR. COLLINS: I'm so interested that

you're going to try to cut up some sort of metrics in terms of the impact of the publication, which, of course, is a topic of great interest in a lot of quarters and everybody will agree to disagree about exactly how you measure impact and whether citations and impact factors are reliable or not, but they're what we have.

I guess, going into that, I'd be curious to know what are your benchmarks? What are you going to consider to be a good outcome? Which I guess would mean you'd want to look at all the rest of the field in terms of CER publications and insist that PCORI publications ought to be at least as good as what the rest of the field is doing, and ideally better. Is that part of the plan?

DR. SELBY: Well, at this point I'll just say thanks for the question. It's an excellent question. It's a challenging question and we will -- oftentimes, I think, trying the very first little bit of it helps you then spot the benchmark rather than pre-specifying it. But we will definitely take that to heart.

DR. COLLINS: If it's worth knowing about, George Santangelo in our Office of Portfolio Analysis has been working pretty hard on how you do this. Actually, our effort is to evaluate the output of particular study sections because you would expect that NIH-funded research is going through rigorous peer review in a particular study section, ought to produce publications that are at least as good as a mean for that particular field. You have to be field-specific because, obviously, impact factors vary a lot amongst different disciplines, and that has been pretty revealing. And it does look as if there are some metrics that stand up pretty well to what is still the gold standard, which is sort of expert opinion, for what that's worth, in terms of whether a particular publication has had a large impact or not.

At any rate, it might be useful to have a conversation with George about the way in which that research has been going and see whether there's a way to apply it in this situation.

CHAIRMAN NORQUIST: Yeah, that would be

helpful. Harlan?

DR. WEISMAN: Harlan Weisman. I'm wondering about internal performance metrics, which I didn't really see. And the context for my question is based on comments that I get as a Board member from people in the research community and also in the patient community -- and I mean this as constructive statements as opposed -- because I think that we have accomplished a remarkable amount -- and that is lag times within PCORI. You know, time it takes internally to process applications, process contracts, the feedback loop back to contractors, comments.

Very rarely do people come to you when things are going great. Most of the things you hear are on the complaint side of things, but that's one of the complaints, if you will, that I hear, that things just take too long internally at PCORI. I have no way of judging that and that's why I'm wondering if we have any metrics around that.

DR. SELBY: We used to have metrics on

there that were sort of pushing the ceiling so consistently that we took them off. I mean, they were like answering the phone within, getting back to people within, and we took them off. But that's not to say that we don't agree. We actually do agree that in the recent past we've had issues around contracting, which Regina in her role as COO is dealing with. So I think -- we'll take that suggestion back and see if there is a metric where we suspect we're not doing as well.

Having said that, you are right that you tend to hear more of the negatives than the positives.

CHAIRMAN NORQUIST: Steve?

MR. LIPSTEIN: You know, Joe, I'm looking at Dashboard and I'm coming back to the pragmatic question we talked about a minute ago where I was trying to understand whether we have greater success as the cycles go on. So, in the broad TFA's, using the slides in the deck, we made 25 awards in Cycle 1, 51 in Cycle 2, and 71 in Cycle 3, and that was in the -- [Sound dropped.] And

then what's interesting is even though your letters of intent come down, because resubmissions are going up, your success rate's greater.

And if you look at the engagement awards, we've had that same track record of improvement. We went from 3 to 7 to 18 between the fourth quarter of 2014 and the second quarter of 2015. So I guess what I'm just suggesting is, is there learnings from our experience with either the broad PFAs or with the engagement awards that could inform some of the challenges we have as we go into the third cycle of the pragmatic studies.

And I understand the concern about the letters of intent going down, and I think Harlan Weisman and Krumholz made an interesting suggestion for more intramural studies, so advancing this is important. But I still think there's some learning here from our previous experience with PCORI that says that not only are we a learning organization, but the research community is a learning research community, and how can we improve our success rate with pragmatic studies, the way we did with the

broad PFAs?

CHAIRMAN NORQUIST: Barbara?

DR. McNEIL: So, two comments. A very nice presentation. The first is, Joe, in your listing of the publications from the past three years or so, I think it might be nice going forward when you actually have a publication that's A versus B, whether it's diagnosis or therapy or whatever, you maybe take those results to the appropriate professional society and ask them for comment about where they think those results are going to be used in changing patient care. Authors always say they're going to do whatever, but it might be nice to have an external group to make a specific comment about that and you can put it on the web.

And the second comment I had related to the dashboard and recruitment. I think it would be nice to know why studies don't start on time and maybe that gets to Harlan's comment about contracts and whatever. Once you know that, I think we really need to have a tough look at delays in

recruitment. We just can't let people not recruit on time without having serious consequences.

DR. SELBY: Thanks, Barbara. I'll just say we actually hope going forward, as the study comes in, the professional society will already be involved in it. That is sort of part of the team of engagement. When they're not, and some of the earlier smaller studies may not have engaged the right professional organizations adequately, I think your idea is exactly right. It would be part of that flow of how do we disseminate and talk to the organization about their take on the findings and what they feel is appropriate.

CHAIRMAN NORQUIST: Let's not forget performance of the actual things. I think that is really key, and we do need to be up front about what the rules are of the game for those who are going to do it.

DR. McNEIL: One comment on that one. The thing I worry about a lot is the ability for investigators to indicate a larger patient population that's available to do studies, that is

really available. It happens all the time, and that makes the calculations look right. It happens.

CHAIRMAN NORQUIST: Well, that's what gets you the funding when you come in.

DR. McNEIL: That's what gets you the funding, you know that as well as anybody. I think that's why we really have to keep our eye on this.

DR. SELBY: I think when we saw these numbers this time and there was this downward trend, we launched a communications internally. Part of it is we don't feel entirely on top yet of how we are capturing the recruitment data. I'll definitely commit to the Board to report back with the results of our inquiries. We completely agree that we have to keep a very close eye on recruitment.

CHAIRMAN NORQUIST: Leah?

MS. HOLE-MARSHALL: Thank you again for the presentation. I think Dashboard, they are ever evolving, and that's terrific. I won't repeat too much of the recruitment conversation. My first

suggestion is maybe the 100 percent, and you alluded to this a little bit, of all milestones is not for the Dashboard. Not being familiar, I would assume there would be more than perhaps just recruitment, although recruitment is a key milestone.

I would just be interested in knowing more from staff about if there is going to be a combined measure that is less than 100 percent of what another key measure is.

I actually do think it is a measure of a successful organization when we can stop things that aren't working, and because there is so much pressure on ensuring that we are funding enough studies, if that bleeds over into oh, we'll just continue it a little bit longer to see what happens, I would hate to see that happening.

I just wanted to say as a Board member I'm very interested in making sure that we do stay on top of it and where appropriate we do stop certain studies, not every single one is going to be successful, so that number probably should be zero

forever, at least from my perspective. That's not a failure, that is actually good management to make sure we have that.

That money can be redirected then into more funding. I'd rather know that sooner if it really isn't going to be successful.

DR. SELBY: Thanks, Leah.

CHAIRMAN NORQUIST: Gail and Bob, if you can keep it brief because we need to move on to the next discussion.

MS. HUNT: I just wanted to add to what Barbara said, and in addition to going to professional associations and asking them about how they would see these findings going out, I think we should go to the patient advocacy organizations as well. They can be very powerful in getting the word out about good results.

CHAIRMAN NORQUIST: I would argue other stakeholders, too, like insurance companies and whatever, how important it is to them. Bob?

DR. JESSE: Just quickly. Having been on the side of studies that had low enrollment, not

for lack of effort on the investigator's part, I actually think it is really important that we not jump into it with an accusatory tone, but rather try to understand what are the issues around the enrollment. In the end, that's really going to shape a lot of how we do studies and remembering that we are dealing with a very different population of researchers in some respects.

CHAIRMAN NORQUIST: And in some sense, different populations. I think that is key. You don't want to cut them off -- I don't think Leah would say -- the point is to try to be as helpful as possible, but there are some times when it is just not going to work and we need to stop.

DR. JESSE: That's true, but don't discourage people.

CHAIRMAN NORQUIST: No, I agree with you. Joe, I think it is now the budget.

DR. SELBY: That's right.

CHAIRMAN NORQUIST: I think Regina and Larry are going to make this presentation.

DR. SELBY: Yes. Larry, you may want to

start off, and then Regina will come on up.

MR. BECKER: Thank you, everybody. I think we have had five months of results. This is our first Board meeting since roughly the middle of the year. Both the FAC, Gary, Bob, and I reviewed this. The chairs of the various committees have had a look at this. I think all and all, it's getting better. We are getting better processes, better understanding of the causals, and as we get better understanding, we will be able to better look at the organization to understand where actions and activities need to occur.

I always think of financially as sort of the canary in the coal mine. As we begin to do this, I give a lot of credit to Regina, as she has pulled this together and continue to improve the process for the entire group, and it is a lot of work, with a lot of projects going on, and it is just continuous improvements.

Regina, thank you on behalf of the committee and everybody else, and we are looking forward to your presentation.

CHAIRMAN NORQUIST: Let's hope the canary is still alive. Regina?

MS. YAN: Thank you, Larry. This is the mid-year review to report to you where we are, particularly compared to what our plan was in the beginning of the year.

I will go over with you the revenue and cash budget and our research obligations and also funding commitments so far. We will also look at our budget versus actuals through February. We will be looking at the various analyses, most of them are in two buckets, the savings side or the activity is delayed, so the expenses actually will be coming in later.

In the beginning of the fiscal year, our cash balance was $626 million. During the first five months, we have received $251 million in revenue, which consists of the Federal appropriations, $120 million, CMS transfer of $92 million, and the PCOR fee of $38.9 million.

Actually, the PCOR fee here is mainly an adjustment in the recognition of revenue because

the majority of our PCOR fee comes in in August. This year we are actually anticipating about $250 million of revenue in PCOR fee.

During the same period, we also dispersed about $72 million. At the end of February, our cash balance was $806 million, $800 million is in the Trust Fund, and the rest is in a bank account.

It may look like we are sitting on a lot of money but actually a majority of the money has been committed to funding research.

Our accumulative obligations in funding commitment is $736 million, and we actually have $604 million of outstanding payments, mainly because most of our projects are multiple year, so we pay them out over a period of years.

Let's look at the fiscal year. We have a plan to fund $640 million in research projects, and as of February, we had committed $64 million, but I also want to point out that at the last Board meeting in April, the Board actually also approved $120 million of research funding, so actually we have committed $200 million so far, as of today,

and we have some coming up today, and we also have quite a few coming up between now and end of the fiscal year.

At the moment, we may be looking at probably close to $600 million of commitments, because in the beginning of the year, we will have a plan, and as the year goes by, and it depends on feedback from the Board, the plan gets adjusted.

For this year, we have an approved budget of $361 million. Our budget through February is $121 million, because a lot of the expenses projections is based on what we project our expenses will be month by month, so a lot of expenses are projected starting in the second half of the fiscal year.

Our actual expenses through February is $85.9 million. Obviously, looking at that, at least through February, we are under spending at about 29 percent.

This is our budget by major categories. If you look at the first line, which is research engagement awards, in that particular line, we

under spend by $22 million, because our year to date through February, our total is $35 million, and the majority of that comes from that one line, and mainly because when we were preparing our projections of expenditures, we were at that time looking at potentially two topics that would be funded through MOUs with agencies and usually with those MOUs, once we sign it, we transfer the payment over and the expense will actually occur at that time.

As time goes by, it looks what happened with those two topics is one, we decided to incorporate it into our pragmatic studies, and the second one is now slated for 2016. There is $25 million right there.

If we remove those two MOUs, the $25 million, the rest of our research spending is pretty much on track.

Secondly, I will go over later about why we are overspending in science development and evaluation and also contracts management.

As far as general administration, we are

more or less on track, under spending a little bit but not significantly.

The last slide shows you where the variance is. This one basically shows where the percentage of the total budget is. For fiscal year 2015 budget as we were planning our spending, we were projecting that for this fiscal year, our administrative spending ratio would be about eight percent. If we look at our spending through February, we are at 12 percent, and last year at this time our administrative spending ratio was 25 percent. We are trending in the direction we were anticipating. I know that was a concern of the Board, so we are pleased to see that operating costs and administrative costs, the ratio is going down.

I want to go over where the under spending and where the budget versus actuals variances come from. Here, we have about $4.5 million which we consider cost savings. One is in the personnel cost side, as we were projecting our expenditures, this year we actually have about 53 new positions

that we are trying to fill, and we were planning the new staff would be arriving kind of on a quarterly basis.

We budget at the beginning of the quarter, and of course, not everybody would arrive at the beginning of the quarter. We do have some savings here, and we also have built in some contingencies that we don't have to use, so we have savings there.

In addition, previously we were not using the competitive LOIs as much as we have this year. We did budget a lot of funds in our budget to pay for all the costs associated with the review. With the competitive LOI, we are actually looking to saving close to $1 million with all the costs associated with that.

In addition, we now have more meeting facilities in-house, so we can accommodate more meetings, so we don't need to rent hotel space or hotel equipment. That also translates to another savings for us.

These are the three major categories in

the savings area, $4.5 million there.

The next one is activities delayed, so we do expect the expenses will be coming in maybe just a little bit later. One is the two MOUs I just mentioned, one is incorporated into our pragmatic studies, and the other one is slated for 2016, that is $25 million there.

The Methodology Committee, they also have expenses that is delayed, I think they just issued a contract for methodology curriculum, and some workshops will be coming latter.

We have experienced about a six month delay in the Pipeline to Proposal program, in setting up all the contracts for the original entities that will be helping us in administering this program. The thing is now that is all set up, the program is going, and I think we recently just awarded a number of Pipeline to Proposal awards of about $700,000, so that is a delay, but it is coming in.

We also have a slight delay in some of our training contracts.

That is about $27.5 million there. These represent the majority of the $35 million of under spending when we look at the budget versus actual.

I want to talk briefly about next steps, things we are working on. One thing is as we are looking at these data, we will be incorporating these data into our 2016 budget development, because now we have a little bit more historical data to look at as far as our cost projections are concerned.

Secondly, with the peer review plan that has just been approved by the Board, we are now trying to operationalize it, and it does mean for all our research contracts that need to go through the peer review process, we will need to extend that contract period for 8 to 12 months. That will have some cost implications for us, so we are now examining it. There will also be cost implications as far as operating costs are concerned because that means we have to service and support these contracts for another year.

As we are planning out and start thinking

about 2016 and updating our cash flow for the out years, we will also be incorporating those factors into our financial planning.

In summary, while we are under spending for the first five months, we see that some costs are being pushed out to later times, and we do have some areas that represent savings, which we will also incorporate those cost savings for next year.

Any questions?

CHAIRMAN NORQUIST: Questions about the budget? Rick?

DR. KUNTZ: It is improved a lot with respect to understanding where things are going. I appreciate the details. It is still difficult for me to look at the cost analysis, I wonder if you could just make a simple graph where you basically graph to 2019 where the forecasted revenue is going to be and where actual spins are, then we could look to see where the gaps are.

MS. YAN: Thank you for that question. That is the one we normally prepare for September, but maybe we can include that next time in the

meeting.

CHAIRMAN NORQUIST: Yes, I think that would be a good idea.

MS. HUNT: About the Pipeline to Proposal, was that the contract that we had to do something around just one area, like the Denver area, and then it was supposed to be expanded to other areas of the country, that was part of the delay?

MS. YAN: Correct. All those contracts have to be signed by the regional entities that are helping us.

MS. HUNT: They are just getting started now? Is there just one regional entity? There was originally going to be one.

MS. YAN: I think now we have a total of five. Because of that, I think we have just recently made a number of awards with their help. I think we are just committing about $700,000 of Pipeline to Proposal awards, most recently.

MS. HUNT: Does that cover the geographic country? We're not just in that Colorado --

MS. YAN: Now we have five regions.

MS. HUNT: That is the whole country?

MS. YAN: For the whole country.

MS. HUNT: Excellent. Thank you.

CHAIRMAN NORQUIST: Barbara?

DR. McNEIL: Thank you, Regina. I just have one question, and maybe it is for you or Robin, and potentially this is a cost saving question. To what extent are the costs associated with this associated with work that is being done by with the Methodology Committee? It looks to me there is probably a huge amount of overlap in terms of development of methods and educational platforms. I don't know if you have thought about that as a financial issue.

CHAIRMAN NORQUIST: Robin?

DR. NEWHOUSE: Some of the members of the Methodology Committee are active in the EDM forum, but at this point there has not been overlap for these budget items. Is that your question?

DR. McNEIL: I'm on that committee as well, and they have a rather robust educational program, which is listed on Regina's slide. It

strikes me we don't need two educational programs in methods development.

DR. NEWHOUSE: You're talking about the contracts, one for CME and one for academic curriculum or ongoing workshops?

DR. McNEIL: No, it's not contracts. I'm talking about work by Academy Health to educate the community, the research is in Academy Health, the vast majority of which would also be part of --

DR. NEWHOUSE: That money does not represent dedicated funds for joint workshops with Academy Health but there would be no reason not to partner with organizations that could assist.

DR. McNEIL: I was just suggesting non-duplication.

CHAIRMAN NORQUIST: I think that is the key issue, are there things that the Methodology Committee is doing that is an overlap and can we can on that. Any other questions about the budget? I think we had one good suggestion about the graph, I think that would be helpful to have that. Bob?

DR. ZWOLAK: Could you describe for us

what happens to the unallocated money in these various silos, does it just disappear back into the giant budget, which I think is the right answer, but should it just disappear back into the giant budget or should we reissue specific calls for new projects within the category, should we use the money that is not allocated for special projects?

At one time, Freda suggested X prizes, or Harlan always has great ideas for new ways to allocate our research funding. What happens to the unallocated money and what should happen to it?

MS. YAN: You are talking mainly about on the research funding commitment plan. Generally, they get put back into the pot for the following year, and another thing is also with research funding generally, the lead time on the planning and whatever we want to do to support something that is more rapid, I think it is up to the Board to look at.

CHAIRMAN NORQUIST: Rick?

DR. KRONICK: Joe presented earlier and we had a discussion of what we could do to increase

the funding for pragmatic clinical trials and that they were kind of under awarded. When you presented the variance, you didn't present that the under awarding of the pragmatic clinical trials is contributing to the variance.

MS. YAN: Yes, that is because our plan is $640 million for this year, and at this moment we are looking at probably $590 million/$600 million, in that area. We do have some funding cycles we are coming under.

DR. SELBY: I actually just wanted to double check with you on this. I think what I said was one of the reasons for the shortfall in commitments in quarter two was we didn't commit as much as we anticipated and budgeted for in the pragmatic, only by a few million, like maybe $10 million or something a little bit larger than that.

That is true, isn't it? It wasn't on the slide.

DR. JESSE: Just a precaution, it used to be if you came in under budget, you were considered a good steward of taxpayer dollars. Now when you

come in under budget, you are considered as having failed to execute your mission. I would just be very cautious when the leadership is going to the Hill, they are well prepared with answers. Every person you are going to encounter is going to have an anecdote from somebody who didn't get money from PCORI and having money sitting on the shelf is going to be challenging to explain, so you need those answers solid.

CHAIRMAN NORQUIST: That's for sure. Christine?

DR. GOERTZ: I think it was about two years ago that we had decided that for our research funding budget, we would start projecting out for two years. I'd really like to see where we are at with our two year projections for our research funding budget. I don't know if you can answer that now, if we could look at those figures at some point.

MS. YAN: We looked at it for the beginning of the fiscal year, for 2015, it was $640 million, the number I showed, and for 2016 at that

time, $400 million.

CHAIRMAN NORQUIST: Roughly $1 billion over two years?

MS. YAN: Yes.

CHAIRMAN NORQUIST: It might be helpful to look at that at some point. Thank you very much, Regina, and your staff, for doing this. Next up is Bill Silberg. For this one, there will be consideration for approval of the Scientific Publications Committee.

MR. SILBERG: Thank you and good morning. I'm going to walk through briefly suggestions we have for improvements to take advantages of opportunities in the area of scientific publishing. This is something PCORI has had an interest in for quite some time, so I'm just going to give you a little bit of background, and then I'll give you a sense of what we would like to have you consider for approval going forward.

As a reminder, and many of you in this room were here at the creation, at the beginning, I don't mean creation all the way back, but this

creation. We have in place a Scientific Publications Subcommittee, which goes back to PCORI's earliest days.

This was developed through a working group process, and it was essentially charged with having oversight of scientific articles developed on PCORI's behalf. That was defined as being authored by PCORI staff, involving members of the Board as authors, members of the Methodology Committee.

These were pieces that would be submitted to scientific journals that would speak for PCORI. You see here what the specific obligations of this group were. There was also a call for development of an internal management process. This was to be a group of staff who would essentially shepherd manuscripts from idea through development, coordinating with the committee.

Just a quick note. The documentation that created this subcommittee defined its purview as being over articles developed for journals that would undergo traditional scientific peer review as well as editorial review. As many of you know, not

all articles that journals publish go through independent outside scientific peer review.

Having been in one of those journals, I can tell you often times you will have internal discussion by editors who will sometimes seek outside comment and sometimes not for editorials or commentaries, for example.

In the years since this was done, we have seen a tremendous ramp up in our activities and our opportunities as many of you are aware, to put the work that we are doing on the record in scientific publications in various ways.

Again, I'm not referring here to the papers that come out of the funded projects, nor am I referring to papers that might be developed by others that would refer to PCORI, and there are dozens and dozens of those as I'm sure you know.

We have seen many opportunities to consider new ways to develop papers by staff including members of the Board and the Methodology Committee that will talk about the work we are doing, the internal analyses we are doing, take

positions on important topics.

There is a much greater volume of ideas, if you will. We also have seen over the last couple of years that we can do a much better job of taking advantage of the time, expertise, and insight that members of the Board and the Methodology Committee offer as members of the Scientific Publications Subcommittee and as we would suggest, a scientific publications committee.

This, we think, will also give us a chance to really improve and enhance the way we plan the development of papers, and make sure that what we are thinking about is aligned with the Board's interest and the Methodology Committee's interest, and that we are really doing things even more strategically than we have so far, and that we can align this with our overall communications opportunities, because as you have told us many times, and we have been very appreciative for the guidance, we really want to look at integrated set of tools that are telling our story to different audiences. This should make that work better.

We also think this will overall help us advance our strategic goals and be much more efficient to do the sorts of things we are suggesting.

Here are the three things that we recommend. The first one is what we are asking you to consider for approval today, and that is reconstituting the subcommittee as a Scientific Publications Committee with a new charter, which you have in your background materials.

That committee, if approved and empaneled, would work with staff to develop and implement a scientific publications planning framework, a scientific publishing plan, if you will. We would also work with that committee to develop and put into place a revamped internal PCORI editorial working group which would do the day to day work with the committee's input and guidance.

Here is a summary of what the reconstituted committee would do. We suggest up to four Board and up to three Methodology Committee members plus the Executive Director or his or her

designee. These numbers are intended to include a chair and vice chair, if the committee so chooses, or if the Chair of the Board so chooses.

Here you see the specific duties. We see this as a really excellent opportunity to provide some very important high level and strategic guidance.

We also think this committee can do some of the things that the subcommittee was not specifically charged with doing, such as providing content expertise as requested much more broadly than we have had before. We have had terrific volunteering of expertise in content areas of information from the current subcommittee, but we would like to see even more of that.

We also think this is a good opportunity for us to get ongoing advice and adjudication if needed on questions of conflict of interest on scientific publishing or helping guide us as we try to take advantage of various opportunities to develop supported theme issues or supported supplements to showcase various publications that

come out of our work.

Here are the next steps. We will ask you in a moment to consider approving the charter. If it's approved, the Board's Chair may appoint the members of the committee as the Chair is empowered to appoint members of certain other committees.

Once appointed, the committee would work with staff, with me being the main support person, to review a publishing plan. With the internal group together, we will then get started. We will be convening the committee regularly for updates and also providing a series of updates to the EDIC on these ongoing activities, as the EDIC discussed this plan quite vigorously.

It's very clear that coordinating that this committee is doing with the overall activities of the EDIC will be very important because you can easily see the overlap not just with communications and support opportunities, but clearly this is a critical dissemination and to a degree engagement opportunity, too.

CHAIRMAN NORQUIST: We will have a

discussion now. Debra, do you want to say anything at this point?

DR. BARKSDALE: The EDIC did discuss this new process and this new charter several times in a couple of our calls. We are in agreement with this plan.

I think it is important to realize that the subcommittee that existed before was doing a lot of work, but it was doing it under the radar, and it had really become, I think, more of reviewing manuscripts, and I think this new process will help us be more strategic in our publication process.

CHAIRMAN NORQUIST: Let's go around for comments. The people on the call, I keep forgetting about the people on the call. I think Allen and Freda are still there. Do any of you have any comments or questions?

DR. LEWIS-HALL: No, I'm good. No questions.

CHAIRMAN NORQUIST: Allen?

DR. DOUMA: I don't at this time either.

CHAIRMAN NORQUIST: Alicia, are you on? Francis?

DR. COLLINS: I agree it's good to have a proactive strategy for this kind of placement of important perspectives. I guess I worry a little bit about what you are going to do to avoid this becoming slow in its responsiveness, because you are proposing a committee with a lot of very busy people, and one can certainly imagine instances where you really have an opportunity that has to be acted on very quickly, and that will happen, either as a response to something somebody else has published or a contract from a sort of major outlet thing, we have room for a comment from PCORI.

How do you proceed in that situation without having your process end up becoming --

MR. SILBERG: It is a really important question. As you know, because we have actually run into this a number of times, Francis, and I know you do, too, I think what we are hoping or we are expecting is that by having a committee that will be more helping us with overall strategic

guidance and really having an internal editorial group that can move quickly and always have a mechanism for developing these things and move them through in an expeditious way, that by the time we get them to the committee, they will be comfortable enough that we won't have to go all the way down into the weeds with developing a piece or reviewing a piece in detail.

We are really hoping to build mechanisms between the internal group and the committee so that we are all on the same page as to what potential opportunities for papers are, and I think one of the tasks will be to come up with some sort of a rapid response mechanism that folks will be comfortable with. That is what it comes down to.

It will be a matter of trust and shared values, if you will, on what is important for PCORI to move quickly on, and what opportunities we may have to let go for another time, but I hear you.

CHAIRMAN NORQUIST: Let me ask you a question. For this committee, it is about internal publications. Could one perceive that this

committee could also be involved in some of the questions we asked earlier about publications that are coming from our investigators or about some of the criteria, for example, and would that be also a vehicle to have that discussion or does that go back to EDIC? I'm not sure where that particular discussion would go.

MR. SILBERG: We haven't gotten into that in great detail mainly because we have made a separation which is rather traditional between the funder's role in overseeing papers that represent the funder as opposed to the funder's role in the development of papers that are independent products of a research award of some kind.

I think it's an appropriate separation. I don't think it's a wall that should never be talked about in some way. I think we have to be careful from not just the philosophical point of view but also logistically, because we have so many independent researchers with awards who will be publishing, hopefully, quite a bit, a very large volume, in many publications, in many journals.

I think it's worth talking about, but I'd have to think a little bit more about how we could create a mechanism that wouldn't just get in the way and slow down good work.

CHAIRMAN NORQUIST: The group might also be a higher level kind of group that could think about some of the issues, open science or some of the other issues like that.

MR. SILBERG: No question. That is what we mean by "strategic guidance."

CHAIRMAN NORQUIST: Okay. I need a motion to approve this charter.

MR. BECKER: So move.

MR. BARNETT: One of the questions is whether or not PCORI has PCORI statement versus if you just want somebody to write on something like open science, and in that case, anyone can write articles or perspectives, and if they are coming out from PCORI, what does that mean exactly. Does it reflect policy. Does the Board stand behind it.

There are also PCORI promotional pieces, too, which can cut both ways. They can be

fundamental pieces that are laying the foundation for what the future of research should be like. I think the piece we did hit that middle ground of promoting the ideas but not being self-serving in doing so but rather provocative with regard to how the future ought to look and what PCORI is doing.

I just want to say strategically, the Board is involved in a lot of things. Is this another one we should take on where there are PCORI statements or PCORI commissioned pieces, and then the question sort of is also what kind of budget is associated with this.

If it is PCORI commissioned, is it staffed, what goes along with that versus Lori and whoever else in their free time is trying to pull together something, just because we are trying to get someone to summarize something that we are doing in a way to get it out there and disseminated.

These are different functions. I just think as a board, the Board needs to be together about codifying something we are already doing and

putting it in print and getting it out there, that requires very little oversight, and I'm not sure actually the Board or this committee has much of a role. That's operations.

If you are going to commission that because you want the staff to get something out that describes what we are doing, you know, I don't know how high that needs to go. If it is actually something around policy, something higher, that is going to have the PCORI name as opposed to describing a program or describing a direction, that is something entirely different.

If you're describing PCORnet, you could ask whoever you want to be involved, but that seems to me operations. If we are going to say there is a policy thing that reflects the PCORI Board and it is a PCORI organizational statement of how we see the world, that seems to be something else.

Again, I think the Board needs to decide if it wants to weigh in on that. I say that being on several other boards, and particularly American College of Cardiology, which all the time is saying

American College of Cardiology says X, along with the American Heart Association, and then other groups, have joint statements, and those are done for a certain purpose.

We would need to decide of PCORI wants to weigh in, do we find partners, and what is it that is furthering our mission by doing it, and is it worth the opportunity costs, because there are so many things going on here.

You can do the quality control, and you may invite members of the Board or not, but I'm not sure it needs to rise to the top. We are the levels of authority here in terms of commissioning these things, and I just don't want to over reach what this committee has to do when there are things, Joe, you can decide, like we are describing a program, FYI to the Board, as opposed to I need your approval, committee, to do everything.

CHAIRMAN NORQUIST: I think this committee could be helpful with delineating what that line is. That could be very helpful.

MR. SILBERG: Absolutely.

CHAIRMAN NORQUIST: Being a member of a professional organization myself, it takes forever to get some of these statements out, as you know, and it would be nice to be much more nimble on that.

MR. SILBERG: That's one of the reasons that I think having an enhanced internal group to deal with many of those as you described them, operational pieces, we want to give the committee and the Board more assurance that we have that covered, and that when we go to the committee in advance to help set what are these tiers and buckets, that we do want to do PCORI policy statement pieces.

That will be much clearer up front as opposed to sending the committee an e-mail and saying we would like you to review the following paper, and have the members of the committee say why are you bothering us. We have to get that out of the way well up front.

CHAIRMAN NORQUIST: Yes. Barbara?

DR. McNEIL: I was going back to one of

your earlier slides, Bill, on the original Scientific Publications Committee, where it says "Approve manuscript ideas initiated by the Board and senior staff." I guess the question there is suppose Rick Kuntz wanted to write an article on EHRs compared to registries and mentioned PCORnet and the potential difficulties in losing EHR data from many sources, as one of several points he was making.

Is that something that would call for approval by this Board, by this committee?

MR. SILBERG: There probably is going to be a number of cases like this where individuals will be writing about topics where PCORI and/or its work might be mentioned in some way, either in a factual sense, a descriptive sense, or to make a point.

The mechanism we have in place for that now really involves the question of whether that is a statement on behalf of PCORI or a statement that any intelligent independent author would make, and that involves the use of a disclaimer.

We may have to look at that a little bit further, but we do this fairly routinely. It also comes up when we have staff who are invited with their content expertise to be part of a writing team and an author on a paper that may or may not have something to do with PCORI, but it's clear it is not a PCORI statement, they are simply content experts in that case.

I think having this structure will allow us to tease that out much more. I think there are going to be many more opportunities to address these questions going forward.

CHAIRMAN NORQUIST: Thanks. I'm going to call for the vote. This is a voice vote. All in favor?

[Chorus of ayes.]

CHAIRMAN NORQUIST: Anyone opposed?

[No response.]

CHAIRMAN NORQUIST: Anyone abstaining?

[No response.]

CHAIRMAN NORQUIST: Okay. Thanks very much, Bill. We will see about appointing people to

this.

Sue Sheridan, who is Director of our Patient Engagement Program, is going to introduce now about the public comment period. We have an open public comment period this morning.

MS. SHERIDAN: Thank you, Dr. Norquist. This is going to be a very brief public comment because we don't have anybody interested here in the room to submit a public comment, and we also have no one on the phone who is registered for a public comment.

I just want to share for those of you on the phone that if you are interested in submitting a public comment, we will have a second public comment this afternoon at 5:30, and you can register on our event page.

I also will share for those of you on the phone if you want to consider submitting a written public comment, that you can do that at info@PCORI.org, and we will receive those and make sure they are sent to the appropriate committee or staff member to answer.

That is all we have.

CHAIRMAN NORQUIST: Thank you. We might want to add that is 5:30 Eastern Daylight Time.

MS. SHERIDAN: Correct, and you can register online or on the event page.

CHAIRMAN NORQUIST: Thank you very much. We have about ten minutes before we break. I will kind of open it if there are other comments from earlier discussions that anyone wants to make at this point. Ellen?

DR. SIGAL: Curiosity about the public comment. We're patient-centered, we are for patients, we are supposed to be reaching out to large grassroots, don't we find it odd there is not much interest in public comment?

CHAIRMAN NORQUIST: Well, I guess someone could look at -- interested in this particular 15 minute period of comment. I think we get public comments all the time in a variety of other venues; right? I think you don't ever want to have a meeting without some public comment.

I hope you would agree, and Bill may want

to address this, too, we get a lot of public feedback, but it comes through a variety of meetings we have, through social networking, through direct comments that are made to our website, things like that; correct?

MS. SHERIDAN: Yes, I would agree with that. I think our public comment period in this particular session is an avenue, but we have created so many other avenues to receive public comment, through our roundtables, we are going to do two roundtables this year with grassroots from all over the United States, and also two roundtables with large patient groups.

We have created those. We were doing our CMA events where we also have the ability to receive questions. We have Twitter chats. We have a lot of Q&As when we have different programs posted on our website.

We continually have a lot of opportunity to receive and answer public comments.

DR. SIGAL: I understand that and agree there is really a chance to get to the entire Board

at one time.

CHAIRMAN NORQUIST: Let me add, Ellen is bringing up an issue that we need to think about how we might use this period. If you have been getting all these, bring some of those public comments back as a summary to the Board at this point, if we need a session like that.

The other thing we did, and we haven't really kind of followed up on this, we had a couple of times where we had folks who came and actually talked to us, and I kind of miss that, to be honest. I think it would be nice to think about how we might use this period in that way. That was very powerful in some ways. I think that also not only gives the feedback to us, but to the broader audience for that matter.

MS. SHERIDAN: Actually, we have been speaking about how can we take a look at public comment, and could there be specific topics we want to address with the public.

CHAIRMAN NORQUIST: Yes, if we are throwing that out, I mean other stakeholders, too,

bring in groups from our other stakeholders and have an engagement with them here with the Board. It is something to think about.

Bob Zwolak, and then Harlan.

DR. ZWOLAK: I don't think we want to fill that air time with meaningless information, but I think all of us are very interested in finding out what those public comments are.

We went from meetings with four webcam's surrounding us and giant screens and huge audiences in different cities in the country. Now, we are sort of sequestered in a smaller room, and we seem pretty secretive.

I think it would be wonderful. If we have speakers, that is obviously primary, but if we don't, I'd love to hear what information you are receiving.

CHAIRMAN NORQUIST: Harlan?

DR. WEISMAN: Just to pile on, I've been concerned about how insular the Board has become. One of the things we used to do in terms of contact with stakeholders was also going out and meeting

stakeholders. Now that we are in Washington, we seem to be planted here.

That was some of the most meaningful stuff I've ever done in my life really, contact with people in the real world. We talk about real world data. We saw real world people, patients, caregivers, clinicians, people involved in health care systems. It was tremendously impactful on myself personally.

I'm not suggesting that we do those again, it's probably not practical, but on the other hand, I think it is important for us to consider how we guard against becoming too insular and Board room like.

CHAIRMAN NORQUIST: Thanks. Steve?

MR. LIPSTEIN: I don't think we are a secretive board. I think we are a pretty open and transparent board. I think the 15 minute public comment segment that we have devoted at our Board meetings has never really been a good opportunity for patients to engage with the Board, especially when we are in Washington. The people who used to

come often were paid representatives of advocacy organizations.

You heard me comment once before there is a big difference between the paid executive from the National MS Society who I support wholeheartedly, and speaking with MS patients.

I just think it was surprising to me a little bit that we are back in Washington, D.C., and this room used to be populated with 50 or 60 people who used to represent the advocacy organizations here in Washington, D.C., and I guess we have given them enough of an opportunity to be heard through various stakeholder forums that they don't feel like they need to come to our Board meetings or they can just listen in.

I would echo what Harlan and Bob said, that patient engagement and stakeholder engagement is still a very important thing for the Board to do. I just don't know that the 15 minutes Board public comment segment has ever been that kind of an activity.

CHAIRMAN NORQUIST: I think it is

something for us to obviously think about. I will put it back to EDIC, since you are about communication and engagement and stuff, to think about how we might do that in the Board meeting. Sue?

MS. SHERIDAN: I'd also like us to think about in terms of patients and public comment, I would have to say that most patients in the United States really don't know what a public comment is nor do they know how to participate in public comment.

I think we need to take a look at both avenues, or do we recreate what public comment is all about to really capture some of the patient community, or like Steve said, think of a more creative way to engage the Board with our patient community in D.C. and outside D.C.

DR. SIGAL: I would say individual patients, an individual may not, but I will tell you in the world I live in, comments from advocacy groups is clearly robust. We get a ton of very informed patient groups giving us information on

metrics and things they are interested in. When we post or others post, we get a lot.

I would agree the individual patient may not, but for groups that are interested in disease specific things, they are very engaged.

CHAIRMAN NORQUIST: I think that is key, around the disease specific, because people get very engaged, and since we are very broad, how do we really engage on some of these topics. It is something for us to pursue, it is a further expansion of our engagement activity.

We are a couple of minutes early, and we will break. For those of you on the phone, we are breaking for lunch for an hour, and we will be back in one hour. Thanks.

[Whereupon, at 12:15 p.m., a luncheon recess was taken.]

A F T E R N O O N P R O C E E D I N G S

[1:16 p.m.]

CHAIRMAN NORQUIST: So before we proceed, let me note that Board Member Alicia Fernandez was not able to join us at this meeting. I don't know whether Alicia's on the phone, though. But she has indicated her intention to recuse herself from the vote on the PCORnet aspirin demonstration project award based on a potential conflict of interest.

And I just want to -- that's going to be our next topic here, about consideration for approval of PCORnet projects. And I want to remind any other board members who may believe they have a conflict and should recuse themselves to let me know and to please recuse themselves from the deliberative discussion as well as the vote, of course. So I guess that could become evidence as we talk about some of the projects.

So it looks like, Rachael, you're going to do this with Joe. So if you want to make some comments before --

DR. SELBY: Well, Rachael will do two

projects which are clear-cut demonstration projects, funded entirely by PCORI, demonstration projects within PCORnet. And we find that these demonstration projects are a real stimulus to problem-solving. They pull issues out of the abstract and into the real issues like contracting between institutions and recruitment, IRB issues.

So Rachael will present two of those through the SOC and the RTC. And then I'll present a third, which is a small project, but it's a collaborative project with the CDC and NIH that will solicit work from PCORnet.

CHAIRMAN NORQUIST: Before we start, I just want to doublecheck and see whether Freda and Allen are on.

DR. LEWIS-HALL: Freda's on the line.

CHAIRMAN NORQUIST: Thank you, Freda.

Allen, are you on?

[No response.]

CHAIRMAN NORQUIST: Okay. And Alicia, are you on by any chance?

[No response.]

CHAIRMAN NORQUIST: Okay. Thanks.

All right, Rachael.

MS. FLEURENCE: Thank you, Gray. So as Gray and Joe have mentioned, we're going to ask for board approval for a number of PCORnet demonstration project. But before I launch into the specifics, we thought it would be helpful to present this slide to just orient you again to the number of demonstration projects that are currently ongoing in PCORnet.

So we have five work streams currently. The first one is the Aspirin Research Demonstration Project, and I'm going to be asking for your approval for that one today. The second one is the obesity research demonstration project. We already received board approval for this in January, so I won't be mentioning this one today.

The third one is the Health Systems Research demonstration project. This also has already received board approval so it won't be mentioned today. The fourth one is the PPRN Research Demonstration Project. I'm very excited

to bring this to you today, and we are seeking approval today.

And then the fifth one is what Joe mentioned, which is the collaboration with NIH and CDC, and it's called Next-D. So this is just to orient you again because there are a number of work streams right now in PCORnet and we wanted to make sure you have the big picture.

Okay. So I'm going to start with the PCORnet Aspirin Demonstration Project, in which we'll ask for your approval for this. So a few things. This is really the first of PCORnet's research demonstration projects. The topic is the optimal maintenance dose of aspirin for patients with coronary artery disease.

It does answer an important unanswered clinical question, one that matters to patients and their clinicians in terms of which is the best dose post-event. There's some statistics here in terms of numbers of 15.4 million patients have coronary artery disease and are on this high dose of aspirin, but it may be associated with a higher

rate of gastrointestinal bleeding compared to the low dose.

The variation in which those patients get is largely driven by practice patterns rather than by the policy of the clinical evidence. So this large, multi-center trial would really be able to provide necessary evidence to establish the relative effectiveness and safety of these two dosages.

But also, and just as importantly, this trial is really going to be able to demonstrate PCORnet's capacity to support rapid and efficient randomized trials embedded in the delivery of care.

And two things to say is that this trial really looks at leveraging electronic health data collected during the delivery of care, and really minimizes the burden of patients and clinicians. So we think there's a number of transformational aspects to this project that could really help us change the way clinical trials are conducted.

I was asked to give a few kind of reminders of how we came to this topic and a little

bit of the timeline. We started working on this fairly early on in PCORnet's life. So as early as March 2014 we solicited topics from the entire network in PCORnet.

We got 41 topics. These were worked on by coordinating center staff as well as PCORI staff, and we came down to a final short list, which was then reviewed by our PCORI advisory panel first as well as by the PCORnet principal investigators.

So after quite a bit of work with prioritization, both groups chose the maintenance dose for aspirin as the highest topic. The PFA was approved for development by the SOC in June of 2014 and then by the full board in July of 2014.

We currently -- the application went under a modified version of merit review that was adapted to PCORnet since this was a limited funding announcement. We've worked very closely with the CTAP as well as with our external merit reviewers. And the selection committee approved this last week, on April 29, 2015.

So this is the recommended project, so

Aspirin Dosing: a Patient-centric Trial Assessing Benefits and Long-term Effectiveness, also known as ADAPTABLE, and the budget amount is $14 million.

CHAIRMAN NORQUIST: Okay. So now we'll have discussion about this and then we'll get to the motion. So Christine, do you want to say something since you're on the selection committee? Maybe you want to say something about this.

MS. GOERTZ: Thank you, Rachael. I think Rachael did an excellent job of outlining the project. We had quite a lot of discussion about this and what it means to be funding a PCORnet demonstration project and how this fits in with other demonstration projects. But in the end I can say that we're fairly enthusiastic about this project and look forward to seeing it move forward.

CHAIRMAN NORQUIST: You had a qualify there. Were there any concerns? Was it fairly enthusiastic or what was their concern? You want to --

MS. GOERTZ: I probably should have used better terminology. I don't think that there was

any concern that was specifically targeted so much at this particular project. It was more about PCORnet in general. And I think many of the concerns that we may have had when we were talking about this were probably incorporated into Rachael's overview this morning about PCORnet.

CHAIRMAN NORQUIST: Okay. All right.

Rick?

MR. KRONICK: Just a minor comment. I think if we're going to get into this mode of potentially having the board kind of approve of studies, maybe a little bit more of a scientific summary, where we'd have like what is the target population? What's the principal hypothesis being asked? Sample size?

It could be two sides, but I mean, it would be better for us to at least look at that kind of summary level of the scientific part.

CHAIRMAN NORQUIST: We'll come back around this way. Bob and then Barb.

DR. JESSE: So I was going to say pretty much the same thing, and I'll be a little bit more

specific. So, for instance, whether it's 325 or 81, if the base size probably doesn't make a huge difference; but if everybody on 325 is also now taking omeprazole, it creates a much bigger difference.

And so really understanding what's the point of the science behind it and what are the potential implications on individuals and health policy would be just help for us to be a little more informed.

CHAIRMAN NORQUIST: Barb?

DR. MCNEIL: Yes. I have two questions. Actually, one of them follows what Rick and Bob just mentioned. But within that general context, I would like very much to know -- since you mentioned that the PCORnet principal investigators were the ones who made the final selection, I'd be really interested to know how many of them were cardiologists. It just seems like a big cardiology problem, or maybe it's not a cardiology problem. But I would like to know that fact, so maybe you know that now.

And the second one I would like to know is given the conversation we had earlier about accrual, what is going to be the mechanism of watching accrual in a site-by-site approach for this particular project?

CHAIRMAN NORQUIST: Okay. So why don't you answer the first one about the number of cardiologists.

MS. FLEURENCE: Yes. So Bob Harrington, who is a cardiologist, is co-PI, and Matt Roe from the Duke -- from DCRI is also a cardiologist. And it turns out that our co-PI on the coordinating center is also a cardiologist. That's Adrian Hernandez. So it is absolutely going to be led by cardiologists.

And we also have strong patient leadership on the running of the trial. We have a heart patient who's also involved with it.

DR. MCNEIL: Can I just follow up on that? Actually, for this claim that it's a problem, which involves downstream effects, potentially cardiac or stroke or bleeding, it strikes me that the patient

voice, while important, wouldn't be the one I would use to choose the topic, if that's what you were implying.

MS. FLEURENCE: It's more about involvement now in the results of the trial.

CHAIRMAN NORQUIST: Yeah. The recruitment issue now, I think. I hope you're talking about that also.

MS. FLEURENCE: Yeah. We're talking more about now kind of standard -- the standard requirements that PCORI research has for involvement of patients in all steps of the research.

Your second question was about --

CHAIRMAN NORQUIST: Recruitment issue.

MS. FLEURENCE: -- recruitment. So the way the trial is set up, and I think this is what Christine was referring to in terms of some of the conversations we had at the selection committee because some of these methods haven't been tested yet in practice, so we're going to be or they're going to be screening using EHRs at all the sites,

identifying potentially eligible patients, and contacting patients directly with the permission of their clinician to ascertain whether the patients want to be involved in the trial.

Because this is going to be done through email, they have other pathways as well of contacting and recruiting patients. They're going to be using mail and also direct contact as it provides location. So they're also accounting for the fact that not everyone has access to internet or wants to be contacting in that form.

So using EHR data to screen patients, to collect baseline data, and then to do follow up on this scale is really one of the transformational hopes that we have in terms of leveraging the data that's being collected on a daily basis without burdening patients and finishing further.

DR. MCNEIL: But just to get to the question about how are you going to monitor site by site on accrual. Are you going to pull back on sites that don't accrue?

MS. FLEURENCE: Yes. The coordinating

center is going to be monitoring for recruitment rates and also to ensure that we have the right representation of the population. So yes.

CHAIRMAN NORQUIST: Sharon?

DR. LEVINE: Rachael, what does long-term mean? Is that long-term effectiveness in the --

MS. FLEURENCE: I believe the trial is 30 months.

DR. LEVINE: Thirteen months?

MS. FLEURENCE: Thirty, three zero.

DR. LEVINE: Oh, 30 months. Sorry. Okay. Great. And just a comment. One of the challenges that exists with aspirin, because it's not a prescription drug, it doesn't appear in the pharmacy data. So it really is a matter of tracking back through progress notes.

We've had a lot of challenge in terms of measuring adherence to aspirin therapy because of the fact that it's not part of the pharmacy system. So it's very labor-intensive.

MS. FLEURENCE: Yes. We're that that seems difficult.

CHAIRMAN NORQUIST: So other comments about this particular trial? Yes, Joe?

DR. SELBY: Rachael, I don't think you said that the investigators have -- this is to Barbara's point about involvement of cardiologists -- that the American College of Cardiology and American Heart Association are both involved on the research team. So we have engaged the right clinical specialists, supportive of the trial and engaged.

The other thing, I just wanted to put a slightly different spin on Christine's point or augment it. But I think one of the things we said is that one of the definitions of a demonstration study is that in fact it is riskier than a trial because you are doing things for the first time and you are getting more out of it -- that you're getting a scientific answer, we certainly hope, but you're also learning a lot about how PCORnet can efficiently do trials.

So yes, it is a bit riskier, and we all recognize that. And therefore, the monitoring is

crucial. And did you say anything about the first stage and the second stage of funding?

MS. FLEURENCE: I didn't. What we propose to the selection committee is that the trial would be funded in two phases. So there would be an assessment after probably year one to make sure that, for example, recruitment was happening the way we wanted it to.

And there'll be a formal assessment before we proceed with phase -- the phase two funding. So that's our way to mitigate risk with the trial, is to fund it in two --

CHAIRMAN NORQUIST: Ellen?

MS. SIGAL: Again, recruitment is something I actually know more about than I ever thought I should know or wanted to know. What penalties do you have if it's not recruiting with just how you place this contract, just [inaudible] what you can do? And I just think that a lot of the enforcement or penalties or restoring funding if it's not written directly in the contract [inaudible] ability to do anything?

MS. FLEURENCE: Well, that's a great point, so thank you. I mean, I think we -- like everything with PCORnet, we're going to -- the program staff is going to be heavily involved with the monitoring. But I take your point that we should have this explicitly written in the contract. So thank you.

CHAIRMAN NORQUIST: Yeah. And I think it's only fair to investigators, too, that it's very clear to them what the rules are, you know what I mean, so that they know that if they don't perform in a certain way, that they know that it's over. It also gives them some incentive to get there, to be honest.

Okay. Let's -- can you -- oh, I'm sorry. I didn't see. Harlan?

DR. KRUMHOLZ: I just want to make a comment about the overall issues here. I think that what's important for us as a board is to focus on the process at this point. I mean, there does remain some controversy about this topic or, you know, lots of people could debate whether this is

the right place or whether this is the right question.

I think for us at this time, that's not the level of -- that's appropriate until it's appropriate on the RTC, actually, when we just most recently voted on it. But it was a question of whether or not the process had been a good one, whether it had been fair, whether it had been transparent, whether views had been able to be heard.

And, you know, in this particular case, Rob Califf was running PCORnet, a very experienced trialist who has done a lot of -- he has addressed a lot of different questions, has a great sustainability about science. But again, you know, I felt that it wasn't our role to critique it at this point, or what that may have been -- had a chance earlier when you were eliciting suggestions and comments and so forth.

But what I was most impressed by was that there was a good process here in that it resulted in a trial that you've got strong consensus around

and people are excited about. And, you know, whether that would have been what I would have chosen or whether anyone else on this board might have chosen it I think is a different issue.

So one thing that we should be very happy about is that they've steered through a very difficult process. It's not a -- it's not a threatening trial. But, you know, then I thought in the end, what I mean is it doesn't threaten anyone's vested interests.

But maybe, for the first one, that's not so bad because we have enough issues to deal with. And so finding an area that's not necessarily anyone's pain point -- what I mean by that is no company or particular vested interest is trying to protect an interest -- and let's see if we can run this.

And if this goes successfully, then you'll go to the next, the next, the next. But I was really impressed and proud of the process that you've shepherded through, and I know all the other help that you've gotten, Rachael. And so for that

reason, I was very supportive of what this was.

And I just wanted to calibrate it, I think sort of the thinking and the kind of level of discussion. And at this point, I think we should be focusing on were there any flaws in the process. I can tell you, I don't believe that there were any.

And you guys have done a terrific job. I can't even believe that you got consensus on the topic or that you got everyone excited about pursuing this. I think that's a remarkable achievement, and I wanted to congratulate you.

CHAIRMAN NORQUIST: Thank you. So remind me, now. So what we're voting on is to approve the award. But the award, I mean, as you've set it up, there will be a phase 1 and a 2 so that we know that if it doesn't work after the phase 1 --

MS. FLEURENCE: That's right. So you're approving the $14 million with the understanding that the PCORI program will monitor this in two phases.

CHAIRMAN NORQUIST: Right. So we're

approving the award amount and the process, as we've said. Right?

MS. FLEURENCE: That's right.

CHAIRMAN NORQUIST: All right. So I need a motion to approve.

[Motion and second made off microphone.]

CHAIRMAN NORQUIST: Oh, okay. All right. So I had a second? All right. So this has to be a roll call vote, I think. Yes. Mary and others are shaking their head. So Phil, that's why you're sitting up there, of course. I don't know why else you'd be sitting -- okay. So you want to do it, then? Wait. I'm sorry. Steve has a question.

MR. LIPSTEIN: [Off microphone.]

CHAIRMAN NORQUIST: Make it fast? They're going to make it efficient?

MR. SILBERG: Steve is asking if the show of hands is enough or if it has to be one by one.

CHAIRMAN NORQUIST: Yeah, yeah, yeah, yeah. Of course it -- I mean, it has to be okay because, I mean, you're -- I mean, we can't do it or we'll have to have a discussion about that.

Yeah. Let's just do that.

All those in favor raise your hand.

[Show of hands.]

CHAIRMAN NORQUIST: Okay. We can see everybody now. Anybody opposed? No, I'm going to get to them. Hang on. I'll get to them. Anybody opposed in the room?

[No response.]

CHAIRMAN NORQUIST: Anybody abstaining?

[No response.]

CHAIRMAN NORQUIST: Okay. So everybody in the room, then, obviously, unless I've missed some category, has voted for this. All right.

So Freda, what's your vote?

DR. LEWIS-HALL: I vote for it.

CHAIRMAN NORQUIST: Okay. Allen, I think you're on.

DR. DOUMA: I'm in favor.

CHAIRMAN NORQUIST: Okay. I don't know if -- well, I believe you can. Okay. All right.

DR. DOUMA: Yeah. Rich had to recuse.

CHAIRMAN NORQUIST: Yeah. So that's

approved. Now we're on to the next one. Okay.

MS. FLEURENCE: Okay. So I'll just start by saying I'm particularly excited about this one. This is our PPRN Demonstration Project, and we're asking you for the approval to develop this PFA.

CHAIRMAN NORQUIST: Now, let's be clear. We're not funding a project. We're approval for the development of it.

MS. FLEURENCE: That's right.

CHAIRMAN NORQUIST: Right. Okay. Thank you.

MS. FLEURENCE: So as you know, you've approved already a number of demonstration projects for our CDRNs, which are our large health systems -- so aspirin, the obesity trials, and the health systems work.

But there's really been a feeling that we needed to attend to the patient-powered research networks and provide them with the opportunity to demonstrate their ability to both generate research questions that matters to their patients and to their communities, and also be able to conduct the

research.

So this PFA is about helping or supporting the PPRNs in testing their network and in testing their ability to work collaboratively as part of PCORnet. So what we're asking your approval for are for two initiatives. The total costs do not exceed $22 million for up to nine projects in fiscal year 2016.

The first set are eight smaller projects of up to $2.5 million in total costs over three years, and the second project is one cross-PPRN project that allows every PPRN to participate in. And this is for up to $4 million in total cost over three years.

So a few words about the project scope here.

CHAIRMAN NORQUIST: So wait. Is there some confusion about the subjects? I thought you were going to ask a question.

MR. BECKER: [Off microphone.]

CHAIRMAN NORQUIST: Wait, wait. You may want to use your microphone.

MS. FLEURENCE: It's up to you, Larry.

CHAIRMAN NORQUIST: Up to 24, I think he said.

MR. BECKER: Right. But 8 times 2.5 is 20 million, not 22 million.

CHAIRMAN NORQUIST: Up to 24, I think is what they're saying.

MS. FLEURENCE: Yeah, because we anticipate them to not all come up to the maximum.

CHAIRMAN NORQUIST: But if they did? Anyway, okay.

DR. SELBY: You could commission us to fund this number of projects for that amount of money, recognizing that not everyone could come in at the max.

MS. FLEURENCE: We anticipate that some of the less -- some of the newer PPRNs may be able to conduct smaller studies. So we thought that not everyone would come up to the maximum. But I can see how it's a little confusing.

So just a few words about how this will run. PCORI will invite letters of intent from the

PPRNs that address patient-generated questions. So a really key aspect of this is that PPRNs will be reaching out to their communities, to their participants, to generate critical questions of interest to them.

The potential study designs obviously must be optimal to meet the study question. There's a variety of study designs that might be considered. They're on the slide. There will also be a collaborative component to the PFA.

So it's most simply conducting research or it's help simply conducting research of interest to the PPRN. But it's also really enabling and supporting collaborations between the PPRNs so that they can share resources, share tools, and really accelerate the way they're doing research by learning from what each other is doing.

So they're asked to propose many different ways to leverage the tools and resources that exist within the network and create reusable processes for PCORnet. So the idea is that the tools and resources that will be developed between the PPRNs

will be of use for future research, and that's where we start seeing an acceleration of the ability for patient-powered research networks to conduct this kind of research.

So a few words about the timelines. The RTC approved the concept in February of 2015. The SOC approved the -- endorsed the limited PFA development in March 2015. And today we're asking you to consider the approval for developing the limited funding announcement.

We are on track to release the funding announcement towards the end of May should you approve today. And then in January, by January, we'd be able to ask you to consider the actual award slate.

CHAIRMAN NORQUIST: Okay. So now we're open for discussion about this particular motion. So we will start with Francis and come around this way. Francis?

DR. COLLINS: So obviously, a really exciting opportunity. And it would be great to bring the PPRNs more significantly into the

enterprise. Obviously, a lot of this is going to be read very carefully by potential applicants, and you don't want to inspire them to come forward with things that you really are not going to be interested in.

So just in terms of the way in which you lay out the scope, one of the things you mentioned on your slide is burden of disease studies. And that sounds to me like it could be quite general, and there would probably be every PPRN who would like to say something about the burden of their disease.

So how do you aim to try to be more precise about capturing what you're looking for and not having people spend a lot of time on things that really are not going to be responsive?

MS. FLEURENCE: So there will be a competitive LOI process. But first of all, we'll make sure we're not wasting anyone's time in doing a full application. I think we'll be able -- there'll be a balance between language that allows the more mature research entities to do fuller

research, but also enable the newer PPRNs who may not be as sophisticated yet in terms of the data that they have to be able to propose something.

But that's where the up to $2.5 million comes on, so that we would potentially only fund smaller projects on that basis. But I do get your points in terms of being specific. And in the end, we do want questions that generate evidence that are really going to help the patient communities or the participants in the PPRNs.

So I think burden of disease would rank lower than a question about answering competitive effectiveness questions. So we'll make sure to get the language cleared up in the PFAs.

CHAIRMAN NORQUIST: Yeah. And I think to his point, anyway, that these were selected already in some degree. In fact, they are topic-specific, right, disease-specific, so to some degree they've already been selected on this, or a rare disease.

So I think we also want to be careful because if someone comes forward with a rare disease proposal, that might be something that we

need to consider that, too. Right? Okay.

Ellen?

MS. SIGAL: So Rachel, I think I like this, but I'm not sure I understand it. So in a specific disease setting, would you deal [inaudible] that are important to patients? How would this operationalize those in the specific disease where they're looking for answers?

How would that -- give me an example of the question. Or would it be gathering data that would seek better decision-making on drugs? I mean, again I think I understand it, but I'm not sure.

MS. FLEURENCE: Well, we're really trying to not be too prescriptive and leave the PPRNs the flexibility to come up with questions that matter to their communities. So the language allows quite a range of different study designs on specific questions.

So what the PFA says to the PPRN leadership is, please go to your communities and generate questions, and then choose one to submit

to PCORI that's really -- the answer to which is really important to your community. So that's what we are -- we're being fairly open to what they might send us.

But I think, to answer this point, the eights that will be selected are going to have to be very impactful.

MS. SIGAL: Yes. But also in terms of the data, so there are probably a lot of things that they think are most important. Or sometimes the things that are [inaudible] in the disease setting, it may be too open. I don't know whether some examples that ask for --

MS. FLEURENCE: But after the competitive LOI process where they've send in their questions, they will have to present a research plan as well. And I think part of what we hope will happen in this process is that collaboration between the PPRNs is going to accelerate and they're to help each other really put down a solid research plan.

So for those who are less research-focused and really more community-focused, they now have

access to 17 other PPRNs that can help support the technical aspects of the proposal.

DR. KRUMHOLZ: Let me say that we're not just handing them the money, but they're increasing their purchasing power. So they'll be able to work with others. But it's still going to go through the process. I mean, it's not an automatic, go do whatever you want, but it's about saying, if you can formulate a suitable question and if you can navigate the peer review process of the PCORI, then you've got money in order to -- the money's coming that side, from patients to researchers, instead of the other way.

And even the CDRN is supposed to be working with partnerships with patients. But this is about saying -- the [inaudible] here is going to be with patients. And they're hiring the researchers, but they still have to get approval for what they're doing, and I hope mentorship, with regard to the questions.

Here we've got a formative question. Let's see how we can help you put it in a form that

you might be able to work with others. Right?

MS. SIGAL: This is an example like of a question that they could ask that -- I mean, maybe I'm just not getting this --

DR. KRUMHOLZ: What's the comparative toxicity of two -- toxicity from the point of view of my experience of two different chemotherapeutic regimens? And how do people feel taking this versus that? I get that there's a disease-free survival advantage with one versus the other. But I want to know how people feel. And you say, well, gee, no one's really actually ever studied it like that before.

MS. SIGAL: Theoretically, this is what PROs could do. Theoretically this is what [inaudible]. But we won't have the answer to that. We can get safety data which will be confidential, but they're not going to be able to get the data for that.

DR. KRUMHOLZ: Right. But this group will now have money to say, what if studies would help us answer this question? Maybe the studies are to

develop the means to study this first. But they're saying, no one's studying things that matter to us. And this is something that really matters to us, you know, high-function survival or something. Right?

And so how do we hire researchers to start answering the questions that may matter more to us? And ideally, those will then align with the things that the researchers are also interested in, too. But it just gives them some purchasing power. It doesn't say to them, you're stepchildren. I'm not saying that there's anything wrong with stepchildren, but that there's this --

CHAIRMAN NORQUIST: Yeah. Let's not get into that.

DR. KRUMHOLZ: Yeah, I know. Anyway --

CHAIRMAN NORQUIST: All right. Larry?

MR. BECKER: I think I heard you say that there are 18 PPRNs. Right? So are we comfortable at least putting out what appears to me is nine possibilities, which means the fact that nine will not get a grant, that we won't discourage the other

half?

MS. FLEURENCE: So the second project, which is across PPRNs, actually will allow for all 18 to participate. So that's some of the purpose of the second project. And then for the first one, we're actually asking PPRNs to come in as collaborations. So up to three -- each PPRN will be able to be part of up to three applications.

So this should give plenty of opportunity for every PPRN to -- maybe not to lead, but at least to participate in at least one of the demonstration projects.

CHAIRMAN NORQUIST: And of course, that would depend on the topic because they're disease-specific. So their patient populations may not be relevant to whatever the question is.

MS. FLEURENCE: That's correct. The collaborations, though, will happen not just on the patient participation in answering the study, but also just sharing resources, sharing protocols, sharing tools, sharing --

CHAIRMAN NORQUIST: Oh, okay.

MS. FLEURENCE: So there's all kinds of collaborative aspects to this that are --

CHAIRMAN NORQUIST: And the other thing you didn't mention, it may not be 18 at the time because you're recompeting the PPRNs. And so the number may be a little different when this actually goes into effect. Correct?

MS. FLEURENCE: That's correct. We have out [inaudible].

MR. BECKER: I just didn't want somebody to get discouraged to see the numbers.

CHAIRMAN NORQUIST: Right, right, right. No. Okay.

Bob Zwolak?

DR. ZWOLAK: A very nice presentation, Rachael, also very exciting. My question had to do with your choice of the wording that questions must be generated by patient members. And some of that actually just played out between Ellen and Harlan.

And even Harlan, when you talk about assessment of toxicity, can you help me a little bit to understand the reality of the "must be

generated by the patients"? In real life I understand this to sort of play out to be a collaboration between patients and researchers. But it just seems like a pretty strong phrase to be using.

And the second brief question has to do with the collaborative aim. I think that some of the PPRNs would have no problem collaborating, but others, I think, are more population- or disorder-specific. And will everyone have to have a collaborative aim?

MS. FLEURENCE: Yes. So to your first question, one of the aims in phase one for the PPRNs was to work with their communities to generate research questions. So this is something that should be fairly mature at this point in terms of how they've gone about eliciting and talking to their broader community about the patients that matter to them.

We're open to all kinds of different methods to do this. So when they come in with their LOI and the application, we expect that

there'll be all kinds of different ways that they've come up with their questions. But this is not something that we're springing on them. This is something that they've been working on for a while, and it's important in terms of what Harlan was saying of really giving patients and communities voices to say what matters to them.

And then I do agree there'll have to be some translation into the research. But they should be fairly right by now used to doing this kind of work because it was one of their phase one milestones.

Your second question is collaboration. Yeah. So some of this, my answer to Gray, this is not about two PPRNs coming together to answer the same question using their -- pulling data or anything like that. It's really we're leaving a lot of space for what the collaboration looks like.

So it might be as simple as one PPRN has a full-time statistician and this other PPRN doesn't, but really needs that fast time. So that's just an example of collaboration.

So what we're trying to do is really build a community of PPRNs and not have 18 individual networks that are just doing their own thing. We think that by sharing resources on all kinds of different levels, that's how we're going to really accelerate PCORnet as a PPRN network.

Many of them, if they pull together their resources, are going to avoid reinventing the wheel and really be able to accelerate the work. So the collaborations can take all kinds of different flavors, and we're leaving that open to them in how they come in together.

CHAIRMAN NORQUIST: So the incentive here is for them to be creative about the collaboration. Right?

MS. FLEURENCE: Creative. Yes.

CHAIRMAN NORQUIST: Yes. So we'll see. It'll be interesting to see, yeah. And I think the other issue about the "must" is that whatever the method is to generate it, ultimately the topic should be considered by patient groups to be an important topic. Correct?

MS. FLEURENCE: Right. Yes.

CHAIRMAN NORQUIST: Okay. Bob Jesse.

DR. JESSE: When you talk about demonstration projects, just in the broad sweep, what are we talking about? The reason I'm asking the question is we just talked about a relatively dichotomous and straightforward trial that's going to cost 16 million bucks over a 30-month period. What are we expecting to get out of $2 million projects?

MS. FLEURENCE: So we're expecting to be able to move the answers to the questions forward. So, for example, what Ellen was saying -- if we don't have PROs in that area and that's a critical building block to being able to answer a bigger question, we build that building block. So they're programmed on the scientific aspect.

But there's also, I think -- and that's where the collaborative end comes in -- there's also huge progress in terms of building the PCORnet infrastructure. So having them develop the common tools and resources that they can share, that they

can tap into, and thinking about this as laying the foundation for future research in the PPRNs in phase two and beyond.

So what we're getting is not only answers or beginnings of answers for scientific questions, but also the beginning of this common infrastructure that they can use to accelerate their research going forward.

DR. JESSE: So something like how to do point of service enrollment into a trial would be--

MS. FLEURENCE: Yeah. And for us to be able to host these within the coordinating center host site has all kinds of protocols and processes that help them think, through this agreement, ways to increase recruitment for trials -- all those sort of things that they've been developing as single networks but are now pooling them, these common resources, for the network as a whole.

CHAIRMAN NORQUIST: Harlan Krumholz.

DR. KRUMHOLZ: [Off microphone.]

CHAIRMAN NORQUIST: Oh, okay. Harlan Weisman?

DR. WEISMAN: Along those same lines of questioning of Bob Jesse, PCORnet I always think of as a network of networks. And I'm glad to see that we're involving PPRNs as well as the CDRNs.

I'm still not sure I understand the segregation of CDRNs and PPRNs. And it's also separate but not equal in that we're throwing a lot of money into the CDRNs and a small amount of money and smaller things into PPRNs.

And I'm not sure I understand the theory behind all of this and why we don't encourage collaboration, not just among PPRNs with PPRNs and CDRNs with CDRNs, but across PPRNs and CDRNs to take on questions, and that we're agnostic, in terms of funding, about whether they're a PPRN or a CDRN, but base our funding decisions on the quantity of the proposal.

MS. FLEURENCE: A great point. I'll say that it's not a black and white as maybe my presentation led to believe. So for example, for the ADAPTABLE trial, a PPRN has a highly visible role. It's the Health eHeart PPRN, and they're

actually going to be providing the patient portal for patient input. So I didn't say that in my presentation. But there is a PCORnet community across the 29 networks, and they're working together.

For the PPRN demonstration projects, they're absolutely welcome to reach out to CDRNs. And we know a number of relationships have already formed. We didn't want to put that as an emphasis in the PFA, but if it's appropriate for them to answer their question by reaching out to one of the CDRNs. And then they're absolutely welcome to do that.

We felt that by emphasizing that in the PFA, it leads to other unintended consequences that we wanted to avoid, I think, for this first set of demonstration projects. This is to help them really ramp up and get out of the gate in terms of conducting research, and remembering that all 18 are at very different stages of development.

So for some, it's going to be a lot easier. For others, they're grassroots networks

that just really need to organize at this point. So we felt that this was the right way to peg the amounts and the message. But we do think of PCORnet as an overall community, and I think we'll see that in some of the LOIs that come in.

CHAIRMAN NORQUIST: Joe, did you want to--

DR. SELBY: I just wanted to be clear, Harlan, that in phase two, the expectations on PPRNs and CDRNs are that they will work together. That is really very clear in the funding announcement and will be very clear in the awards. And we're looking for what they tell us about how they will do that, and there will be milestones.

So we heard the board loud and clear. We had not put it that clearly, particularly in the funding announcement for the CDRNs round one. So I think that's really a fundamental aspiration in phase two, is to get them much more integrated.

CHAIRMAN NORQUIST: Ellen?

MS. SIGAL: Again, I'm struggling between being too prescriptive and being outcomes-driven. It would be great to have specific questions that

you think -- or examples of questions because I can -- and of course I have to think through the lens of the community that I live in in cancer.

And obviously, Harlan, your question on toxicity could take years, and it would be irrelevant by the time you ask the question on it, PROs or something that's been in development. And we still can't [inaudible] in a meaningful way.

You know, we've expanded access to something people care about, but you're not going to solve it. So I'm still trying to figure out this kind of question that we meaningfully ask and get answers to. And I'm struggling with it.

I think it's a great idea, but I'm trying to think about, just as an example, that would then maybe lead people to begin -- because you're going to get a lot of staff, like access to clinical trials. I mean, how much more can we do on that? You know, we need to do more, but I'm trying to figure out -- and maybe other disease settings, people can get ideas from [inaudible] a little bit.

MS. FLEURENCE: Yeah. So I think, Ellen,

I mean, a large -- I mean, we expect to get a fair amount of just traditional CER questions that do competitive effectiveness between treatments. The amounts that are proposed are the amounts that the CER program that PCORI gives out. So we expect to get a fair amount of traditional CER questions.

Since some of the networks are very grassroots and have just got out of the gate, we wanted to open the door to have some message projects come in as well. So, for example, PRO development could be one question.

So I think the PPRNs will be fairly creative in just coming up with the questions with their communities. And we'll make sure that these are answerable questions in the timeline and the funding that we provide them.

CHAIRMAN NORQUIST: Harlan Krumholz.

DR. KRUMHOLZ: I'll just say I think that the premise is that the researchers and the content experts of a particular field don't have a monopoly on what the good questions are to be asked, and that the questions of patients or those who are

experiencing these conditions have maybe raw, maybe not in the form of a scientific question yet, but may serve as the guidance for it.

And the question is, if you give them purchasing power -- it's an exempt, by the way. It's a risk. How is this going to work? But as long as they didn't have any access to capital, they had no ability to -- they had no power, really, to be able to pursue their questions.

And I don't know -- with regard to toxicity, if that ends up being a good question, and there are tens of thousands of people being treated and they say, what we want to know is what's the difference in terms of functional status -- nausea, concentration, sleep, taste, a whole range of things that people aren't collecting -- then maybe those can enter.

But it's an exempt, for sure. But it's one that I think that I for one would like to see us pursue and to see, what do they come up with? And again, they're going to have to be mentored in the sense of being able to configure it in a way

that it can be tested formally using the scientific method.

But with regard to the question, I expect them to have more expertise in themselves and what their experiences are and what are the things that they might wish that someone had answered. And when they ask their doctors about it, they say, you know, no one's really studies that very well.

And for anyone who's got a chronic disease, I think there must be millions of questions that we just gloss over but actually importantly impact their lives. And they wish that somebody could have a little more evidence thought might help them make the more informed choices, knowing what their experience is going to be like with those choices.

CHAIRMAN NORQUIST: Yeah. I think this is going to -- a very good argument. I think it's going to be an experiment, we'll see, when you bring these groups together with some science to see if they can come up with a question. So we'll see.

Freda, did you want to say anything? Because you come out of the RTC, and I just wanted to let you say something.

DR. LEWIS-HALL: No. I think it's been a really robust -- every time I wanted to raise my hand to say something, someone else made the point, I guess.

CHAIRMAN NORQUIST: Okay.

DR. LEWIS-HALL: It was a great conversation.

CHAIRMAN NORQUIST: All right. Allen?

DR. DOUMA: I'd just like to reinforce what Harlan Krumholz is saying. It is an exempt, but it's one that we can take and others can't.

CHAIRMAN NORQUIST: Okay. Thanks. Any other --

[No response.]

CHAIRMAN NORQUIST: Okay. So I need a motion for approval. And remind me, so this is -- it says limited PFA funds, up to nine. And I think we need to say the amount. Right? Because that's missing. We're also approving, right, up to nine,

for a total funding of --

MS. FLEURENCE: Twenty-two million.

CHAIRMAN NORQUIST: Okay. Twenty-two million. Okay. Research demonstration projects within PCORnet. And then we'll see what we get. Right?

MS. FLEURENCE: That's right.

CHAIRMAN NORQUIST: Right. Okay. Yes. Sharon, you're making the motion, and then the second?

[Motion and second made off microphone.]

CHAIRMAN NORQUIST: Thank you. Okay. We'll do it again with a show of hands. So all those in favor raise your hand.

[Show of hands.]

CHAIRMAN NORQUIST: All those opposed?

[No response.]

CHAIRMAN NORQUIST: Anyone abstaining?

[No response.]

CHAIRMAN NORQUIST: Yeah, Sharon. Yeah, I was looking. She did raise her hand. So it was unanimous in the room. Thank you for that.

And then so I need to hear from Freda.

DR. LEWIS-HALL: Approved.

CHAIRMAN NORQUIST: Allen.

DR. DOUMA: Aye.

CHAIRMAN NORQUIST: And I don't know if Alicia is on the phone. I don't think she's recused.

[No response.]

CHAIRMAN NORQUIST: No. Okay. So it's unanimous of those who are there. And that was the same for the last vote also.

Okay. Thank you, and we're on to the third. Is this yours, Joe?

DR. SELBY: Yes. So this is an interesting study where the idea really came to us from the CDC. So the program officer at CDC in the Division of Diabetes Translation called and said, you know, we have a project called Next-D. It's a five-site, CDC/NIDDK-funded, multi-center study just completing its first five-year cycle. And it's going to be recompeted.

The aims of Next-D from the beginning have

been to study natural experiments, particularly within systems. There's not a lot of data-sharing or, you know, cross-system work, as I understand it, among the five systems, but the variation in policies and clinical intervention that systems mount to prevent or manage type 2, the most common, the more common, type of adult onset diabetes.

So these are natural variation, natural experiment kind of studies. They are by definition comparative effectiveness, and they are all observational studies, to my knowledge.

CDC invited PCORI to join with them and NIH in helping to co-fund a second cycle, with the express and sole intent of adding some CDRNs. They knew of PCORnet and they like the idea of involving some CDRNs in Next-D in round two.

So adding PCORI funding would allow them to increase the number of sites funded from five, as in round one, to up to eight. We'd provide the funding for the additional three. Now, what CDC funds is $450,000 per year for five years for each project. So that comes to just under $7.5 million

to PCORI if we funded up to three CDRNs to participate in that.

Our rationale for thinking positively about this started with the fact that we have a policy called our principles of collaboration which says we look for opportunities to collaborate with other funders and to co-fund. This also allows for collaboration among some PCORnet sites.

The CDRNs could -- even though maybe the rest of Next-D's awardees don't pool data in any way or conduct joint analyses, the CDRNs could because they share a common data model. But there's other features that also appeal to us about this.

One of the key ones is that almost unbeknownst to me, an internal network, internal to PCORnet, had sprung up of six CDRNs who are already studying type 2 diabetes. And so there were ongoing meetings between these six CDRNs about building the capacity to do diabetes-related work in PCORnet.

Another thing about Next-D which I like

and is very consistent with PCORnet is it pushes the awardees to be in touch with their delivery sites and their plans just so that they are aware of the natural experiments thought are going on and they join with their systems, their delivery systems and plans, in evaluating that care.

It's also a chance to demonstrate and expand PCORnet's standards and methods to accommodate a model to the other systems and researchers in Next-D. And it gives PCORI and the entities participating in PCORnet some experience that we haven't had a lot of yet in analyzing natural experiments from delivery systems. So this observational analysis of things that help systems do every month and every week is something Next-D has got a real methodologic expertise in and we could learn from.

Here's the way it would work. The scope would be that we'd fund up to three CDRNs, beginning in fiscal year 2016, so beginning October 1. That's when Next-D kicks in. It would be approximately $1.5 million per year in total costs

-- actually, just a little bit less than that -- for the three sites for five years, a total of just under $7.5 million.

The way we would do the competition is the PCORnet CDRNs have in fact applied to the CDC. Five applications were submitted by PCORnet CDRNs to the CDC, and they will go through the CDC's merit review process. PCORI would then further review applications with the technical review scores from the CDC in hand. To ensure comparability with the way that awards are made at CDC, PCORI would have the technical scores from those awards.

Applicants would have to apply with PCORI's additional requirements for methodology standards, adherence to methodology standards, peer review of publications and early release of publications, patient-centeredness, and engagement, which I think those two latter ones are pretty baked into Next-D already. Collaboration with CDC and NIH will also take into account our collaboration principles, and they will comply in

every way with our authorizing legislation.

One thing I didn't say yet -- it's not on here -- but it's quite possible that one of the CDRNs from PCORnet could compete successfully and be in the top rank of scores at the CDC, in which case the CDC will be bound to fund it. The CDC will fund it itself. So it's conceivable that we could wind up funding up to four CDRNs, assuming they do well on the technical scores at CDC and if the CDC should fund one of them.

So the proposed timeline is that we presented this to the SOC on March 17th, and it was endorsed for development of the PFA. We presented it to the RTC eight days later, and it was again endorsed for the same purpose, to develop the PFA. Today we're talking to you, and we hope you will approve development of this PFA.

In June we would release the PFA. The review would take place over the summer. And we would hope, although there is room for a little slippage of up to a month or two if the review process winds up taking that long, but we would

hope that in September or no later than November we would come back to you with a recommended slate of from one to three awards.

That's it, I think.

CHAIRMAN NORQUIST: Okay. So we have some -- I just want to -- I'm trying to be sure I've got this right. So the CDC's going to commit to funding five of these. And then we might fund one, up to three. But when you were talking about the extra review or something at PCORI, does that apply to all seven or eight of these, or just the ones that PCORI is going to fund?

DR. SELBY: No. Just the ones that --

CHAIRMAN NORQUIST: So the CDC-funded ones might be a little different in some way, theoretically. So how would that part --

DR. SELBY: Well, they are different from each other. And --

CHAIRMAN NORQUIST: No. I mean that apply or that we're holding them to.

DR. SELBY: They will be reviewing for technical standards -- you know, more traditional

research team, the strength of the analytic methods. They would review for that. We will review for adherence to our methodology standards for patient-centeredness and engagement only.

CHAIRMAN NORQUIST: Just -- I don't know. Somehow, at the end of it, something seems odd, that there's one or two or three that are going to be different in some kind of -- essentially different somehow. So maybe I'm just --

DR. SELBY: Well, one thing I'd emphasize is -- I said it before, but I think it might be helpful to say it again -- the aim of Next-D is not so much to pool data and do everything the same. Next-D creates a forum where people that are interested in what systems are doing about type 2 diabetes convene, compare notes, think about studies that could be done.

You know, a person from one site may suggest a study that could only be done in another site. But it's an intellectual focus. It's also a place where methods for this kind of research are discussed. But the five or eight -- five to eight

sites don't have to be entirely consistent, and they aren't doing the same evaluation project.

CHAIRMAN NORQUIST: Let's go around. Francis?

DR. COLLINS: I just want a little bit more clarification about actually the scope of what's being proposed here. This mentions natural variations within different systems. So this is not an interventional study; this is observational of different systems?

DR. SELBY: It is.

DR. COLLINS: And it also mentions here both preventing and managing diabetes. So is some of this based on identifying people with pre-diabetes?

DR. SELBY: One of the projects that I'm familiar with within Next-D works with a health plan that is collaborating with employers to identify people who are at high risk for developing type 2 diabetes, and does essentially work site wellness programs on them.

So yes, some of it is about prevention as

well. Another one is a health coaching project in the same population. So yes, they actually are -- health plans are very interested these days in the pre-diabetic state, which you can recognize pretty easily and then intervene before --

DR. COLLINS: With that data from the BPP to what can actually happen --

DR. SELBY: Exactly.

DR. COLLINS: But there's no effort here to try to convince the applicants to do something different than what they're doing. Rather, it's to observe what the outcomes are and make a comparison.

DR. SELBY: Exactly. It's trying to learn what -- it starts from the premise that health systems are doing a lot of things. These are, in effect, natural experiments and their impact is not being captured.

CHAIRMAN NORQUIST: Okay. Debra, you had your card up first during the conversation.

MS. BARKSDALE: Yes. So help me understand, and I think some of this is in line

with the questions that Francis asked because I'm not quite understanding how all of this comes together. And these -- yet sites have been funded for five years prior?

DR. SELBY: Yes. They have been underway for five years.

MS. BARKSDALE: And that there's some impact? Or what -- I guess I don't understand what the impact has been over the five years and what we would actually be contributing to that might be different or enhance the [inaudible].

DR. SELBY: Well, I think that, you know, given the nature of the Next-D collaboration, which is to meet periodically to keep each other abreast of the activities that the individual sites are doing, to look for ways to collaborate, both on methodologic questions and possibly on comparative effectiveness, questions -- you can jump in any time to this. And that was CDC's sense.

You can jump in with your experiments and your systems at any time and begin discussing and looking for opportunities to collaborate. I'm sure

that some of those studies are done. I haven't -- you know, some of the things that they have done in Next-D are completed by now. They're basically four-plus years into it already.

But I don't have a list of the applications for -- of the publications. I can tell you I know that each of these sites does collaborate closely with the delivery system it's affiliated with. So I would expect that one of the impacts has been that the delivery systems have gotten to know something more about their own data and what they were doing.

CHAIRMAN NORQUIST: Christine? Wait. Is there a question?

UNIDENTIFIED: [Off microphone.]

CHAIRMAN NORQUIST: Yeah. All right. Christine?

MS. GOERTZ: Thank you. I just have a couple questions. I wasn't actually on the call when the SOC talked about this. So how many of these are funded now?

DR. SELBY: Five.

MS. GOERTZ: So is the thought that those five would be re-funded and plus an additional three? Or how --

DR. SELBY: It's a competitive reapplication process, and the CDRNs entered that competitive reapplication process. I mean, you know, the --

MS. GOERTZ: So there were five that were funded. Now it's anticipated that eight will be funded?

DR. SELBY: If PCORI -- if, number one, PCORI gets involved; and number two, if PCORI -- after PCORI's review, there are three CDRNs that look like they've done well on the technical, and they also meet our additional criteria.

MS. GOERTZ: So I have a little bit of concern about the review process as to how that might work because how you might write an application for the initial review may not be the same way that you would write an application in order to meet PCORI's criteria. And because you have limited space, you're going to have to decide

what to emphasize.

And I'm just wondering if it might be more fair to investigators to allow people to come in and add PCORI criteria, or after the first group you would have in there -- so I don't know. But I think otherwise it might get a little bit messy.

DR. SELBY: Yeah. Well, that's an excellent point. You know, we are releasing a separate PFA. We're just saying that we're not going to re-review the technical aspects of what you propose.

But it would be -- but they will have to submit something, and I think you're totally right that they can then submit, particularly in the area of patient-centeredness, engagement, adherence to the methodology standards, those kinds -- those points that we're going to then review on. But I get your point. I think it's a very good one.

CHAIRMAN NORQUIST: But let me just add, you know, this is what I was trying to get at. So it sounds like, though -- but it would be -- the five top ones are going to be funded by CDC. And

what's left over is then going to have to compete for ours. Right? So we won't necessarily get to us the top five. The next ones down --

DR. SELBY: Right.

CHAIRMAN NORQUIST: -- I would assume, right, is the way we want to work.

DR. SELBY: Right, right. But only among remaining CDRNs. You know, and if another applicant from somewhere else in the country that is not -- we would only --

CHAIRMAN NORQUIST: So we're really focusing on -- the only applicant for us would be a CDRN?

MS. GOERTZ: Right.

DR. SELBY: One thing, Gray, that I didn't mention but I think some people in the crowd might be wondering about is this will actually -- we talked at length with the CDC about this. And actually, no funding will change hands.

So we will have an MOU with the CDC and NIH. But there will be no exchange of funds between us and the CDC, for example. Those awards

that we would make would be directly made between PCORI and the three sites.

MS. GOERTZ: Is there some guarantee, then, that they'll be treated exactly the same? Because, you know, the person -- they have different masters.

DR. SELBY: Yes. Our Improving Health Systems group, led by Steve Clauser, has been in these discussions. And they will participate in overseeing these, so I think they will get fair reviews. And our three will be overseen by us. So I think it will be fair to them.

You know, maybe our oversight will be different than the CDC's oversight of those five. But I think that's a really small likelihood that that could create a problem.

CHAIRMAN NORQUIST: Gail?

MS. HUNT: Yeah. Gail Hunt. What about when this -- is this going to get down -- these studies going to produce data that's going to be actually useful to caregivers and to patients? In other words, are they actually -- are we getting to

the level of like the primary care doc?

I know a lot of them that deal with employers and wellness programs. But what about the goals of the patients in these projects? And like as in patient-reported outcomes, are there going to be data collected and hopefully disseminated and then implemented based on these five studies?

DR. SELBY: So my best answer to that, and I have to really sit and talk to the CDC about it, but the amount of money that the CDC puts on the table is relatively small, $450,000 per year per site. So I'm not sure how much primary -- you could certainly do some primary data collection for that, some PRO collection, let's say.

I do think that at least to this point, they have taken the tack of trying to answer questions that health systems have. Are these work site wellness programs really working? And CDC was very clear, though, that the CDRN sites could come in with whatever particular topics they were interested in. And in fact, they could get down

and be more clinical.

The other thing I think is that putting these CDRN diabetes interest groups together could spawn a lot of additional interesting topics that they could pursue together using other funding sources.

CHAIRMAN NORQUIST: Okay. Leah?

MS. HOLE-MARSHALL: Leah Hole-Marshall, board member. So my question is about the cost proposal. The CDC is funding $500,000 per year, and then a separate coordinating center for 250. We're proposing to triple that for CDRNs when our purpose of those is to reduce research costs.

DR. SELBY: I'm sorry. We're not -- we're proposing to fund them at exactly the same amount as the CDC funds theirs. The 1.5 was for three of them. The 1.5 million per year was for three of them, for five years.

MS. HOLE-MARSHALL: Per?

DR. SELBY: 500 per year times five years.

MS. HOLE-MARSHALL: So it would be the same cost as the current --

DR. SELBY: The others.

MS. HOLE-MARSHALL: -- other centers.

DR. SELBY: Right.

MS. HOLE-MARSHALL: So my second question, then, is would there be some kind of analysis that our coordinating center could do or that these sites would be expected to do based on their CDRN infrastructure that looks at -- I mean, one thing I would be interested in figuring out is if there's already this collaboration with these five through NIH and CDC, if we're using CDRNs and we're trying to figure out if CDRNs are actually producing faster, different, more efficient research but we're funding them exactly the same, it would be interesting to know if our premise is true and that they actually did have some kind of benefit, even if it's not cost-effectiveness for this one, over this other model.

So is that a part of it? Is looking at -- particularly do the CDRNs do this in a way that produces a better outcome, whether -- I mean, it's not going to be cost since we're already saying

that we're going to fund them the same.

DR. SELBY: Good question. So in other words, given comparable costs, can they do more because they're organized efficiently?

MS. HOLE-MARSHALL: Right.

DR. SELBY: Yeah. But you know, I think one thing is that the other sites are mostly -- they're single site, too. And so they may have efficiencies within their single site already that would be comparable to the efficiencies PCORnet has within its single sites. PCORnet really gains when it combines data across sites by virtue of the standardization.

So we'll have to see. I mean, one thing, as I mentioned, that we could see is that the three funded CDRN sites might do a project that was together across three sites. That would allow for some really interesting comparative effectiveness natural experiments if the three sites do things differently that nobody else in Next-D could necessarily pull off for lack of standard data.

MS. HOLE-MARSHALL: So could we add an

evaluation criteria, then? I understand that we're not going to prescribe what it is. But can we add a criteria that someone is actually looking at this so that we can learn from it?

DR. SELBY: Yes. Thank you. We got that.

CHAIRMAN NORQUIST: Okay. Barbara?

DR. MCNEIL: I'm a little confused, and I think I may have missed the phone call as this was discussed at the SOC as well. And I'm a little concerned because the kind -- I pulled up what the SOC does on the web now and I'm looking at the size project.

And it looks to me as if the SOC spent a lot of time in the past three weeks looking at the kinds of projects that were in or outside that particular -- our particular subcommittee, with particular emphasis on whether we were truly looking at A versus B in the context of healthcare systems.

And I don't know if Steve is here, but several people on that phone call, including Rick and Christine -- you were on it or you were not? I

can't remember. Alicia was. And when I look at these -- the list of the five current projects being funded by the CDC, most of them would admit they do not meet our criteria for consideration as A versus B in the healthcare systems component of PCORI.

So I'm a little confused because when we talk about this tomorrow, all of these would have been in the category "don't fit," or I think almost all of them, certainly four out of five.

For example, the one from UCLA evaluates the effect of the health plan design to reduce costs or out-of-pocket costs. Another one's looking at the effectiveness of EHRs. Another was a diabetes prevention program in YMCAs.

So none of these are really A versus B in the way that you asked us to look at them in our subcommittee, or Christine did, several months ago. So I feel like I'm just at a loss to know how to evaluate this, given what I assume -- what met with the unanimous approval of our subcommittee and which we'll bring before the full subcommittee

tomorrow.

DR. SELBY: These are -- I mean, the name of the study is "Natural Experiments In," so by definition, they all have comparators. Now, the comparators -- I mean, when you say "evaluate the effectiveness of," I don't know how you evaluate the effectiveness of without a comparator. So they do have comparators.

They may well be usual care, and I don't have the details to tell you how they characterize usual care. We still subscribe to the notion that the right comparator sometimes is usual care, and I think you do, too. I'm pretty sure you do.

So I would say that that's exactly what they're doing. So I would really disagree with you. These are very thoroughly comparative effectiveness studies, although the comparator is -- and I don't know, although the comparator is at least sometimes, I'm sure, what they were doing before or what they are doing in some other sector of the organization or with some other employers that don't have a wellness program.

MS. HOLE-MARSHALL: So if we're doing an additional PCORI review for the CDRNs, one of the things -- and it seems to meet our criteria, so as we are providing additional guidance about especially improving health systems and appropriate comparators, that would get into your review. Right? And so they'd still have to come up with questions that met PCORI requirements.

DR. SELBY: Yeah. I think --

MS. HOLE-MARSHALL: And maybe they have a couple, and some of them do meet them and some of them don't. Is that --

DR. SELBY: No. I think --

MS. HOLE-MARSHALL: That's why we're doing the extra people review.

DR. SELBY: You're absolutely right, that we can make it clear. I think -- you know, we've had to communicate a bit with these CDRNs to make them aware of it and to let them know that this is going to have to meet PCORI's usual criteria for funds.

So I think they can -- I haven't heard

anything about this, but my suspicion is they knew this as they prepared their proposals. But we can certainly speak to that in the PFA that we put out, and we can also speak to it at the time we review them and negotiate with them.

CHAIRMAN NORQUIST: So we've eaten 15 minutes into our next topic, so we need to move on. We didn't expect to take -- yeah. Okay. Rick?

MR. KRONICK: I'll be very brief. We have talked many times about the difficulty of demonstrating what research done differently means. And it seems like we have a potentially good opportunity here to say something about that; maybe not.

But we've got a preexisting set of projects that were funded years ago, before PCORI had research done differently. And now we're going to have three new projects under PCORI's standard. And Gray, to your earlier point and kind of Christine's point, it would be very interesting to know whether these three projects under PCORI's standard, in what way they're different from the

five other projects. And I hope we will get a report on that at some point.

CHAIRMAN NORQUIST: As part of the evaluation. That's part of what Leah was also also saying, I think, too.

Harlan Weisman?

DR. WEISMAN: I'm still struggling a little bit getting it. And with each time I heard people's questions, I thought, oh, they're asking the question I would have asked, so I'm not sure I can do a better job.

But Joe, if you looked five years down the line, and we funded these over -- and we're looking at the results, what are we getting? What does the public get in terms of patient-centered outcomes as a result of the outcomes research, as a result of us having done this? What will be different? What will we have learned, not specifically but generally?

DR. SELBY: I think we will -- for a disease that we're a little underrepresented in, given its prevalence and impact, type 2 diabetes,

we will have learned -- and as you say, we don't even know what's going to be proposed yet -- but assuming that we fund two or three networks, we will have learned about the comparative effectiveness of various systems' approaches from the perspective of, among others, patients, measuring outcomes that matter to patients.

So I think, you know, this always comes up with assistance. Our legislation says, answer the questions of patients, caregivers, clinicians, and systems. So in one sense, we'll have information for systems. I think in another, we'll have information for patients.

So my system, my health plan, is calling me to get involved in this self-coaching thing. Does it work? So I think we'll have information useful to delivery systems, and we'll have information useful to patients within that.

DR. WEISMAN: Since there's a number of different experiments, five to eight, what will be the generalizability of the findings of the individual ones? Would somebody on the outside be

able to look at it and say, I've learned something about the management of diabetes in my health -- in health systems in general that leads to changes, improvements, or something, when you have this disparate group of findings?

DR. SELBY: Again, that is the question, really. When you have usual care as the comparator, have you done any more than just evaluate the possible impact of the intervention at that one single plan?

And that's why I think we're coming around to say you had better not only describe usual care but monitor it as carefully as you do the intervention so that in the end you can say, compared to what? Compared to something that we can determine? Is this comparator like what we do in, you know, some outside system?

Otherwise, it is tough to generalize if all you've done is just evaluated the impact in a single system. It varies from project to project, but it's a real risk with that kind of research.

CHAIRMAN NORQUIST: All right. Larry?

MR. BECKER: So I guessed this. So I contract with four different health plans. They all have an approach to disease management. Diabetes is one of the big ones for us. So hopefully, we would be able to look at the various programs they offer, have some basic guidelines, and be able to standardize the approach. Is that where -- I mean --

DR. SELBY: That is where a substantial amount of this was coming from. The plans that were participating who were behind the researchers were saying, we're doing an amazing amount in response to employers' requests. You know, employers really require a lot, and the plans jump when they -- and, you know, one employer requests one thing, and another employer requests something different.

So there was a big cry at that time for, you know, what actually works? What do we know? And I think we don't know a lot about work site wellness programs and their generalizable effectiveness yet.

MR. BECKER: And I would say that, you know, Blue Cross offers it one way, and Kaiser another way, and Aetna a third way. So from my perspective, getting some guidelines and some basic approaches so across my population it's similar would be helpful, too.

CHAIRMAN NORQUIST: Okay. Christine, you have the last word.

MS. GOERTZ: Thank you. I can understand the benefit to the CDRNs about, you know, joining another network and the potential lessons that could be learned from that experience. But I'm wondering -- and Rachael, this is probably a question for you about their capacity, and do they have the capacity, you know, to fully engage in two different networks that may not completely have all of the work aligned? And how are we going to monitor that or triage that?

MS. FLEURENCE: Yeah. So that's a great question. So we'll get some indication of that in their specific applications. But I will say CDRNs have to be thought of as really large groups of

investigators that are organizing. So certainly we have one PI that we work with, but they do -- they are in themselves big networks. So we won't necessarily get the same PI leading the Next-D initiative.

So I'd say that -- I mean, these are pretty solid networks with [inaudible]. And so we see that, I think, across all of the CDRNs. So I would say that's less of a concern from my perspective.

DR. SELBY: I think this is one of the first that, you know, tests the notion that the CDRN itself can be a resource that other investigators in the communities can work with and bring their expertise to.

CHAIRMAN NORQUIST: Okay. So it says here the SOC and RTC apparently approved this, whoever was on the call. And so at this point I'd like to ask for a motion to approve this. Second?

[Motion and second made off microphone.]

CHAIRMAN NORQUIST: Okay. I realize we didn't -- Freda, if you had any comments you wanted

to make on this since this came out of the RTC also.

DR. LEWIS-HALL: No. You guys were on a roll.

CHAIRMAN NORQUIST: Okay. So we're going to do it again with the hand vote in here. So all those in favor?

[Show of hands.]

CHAIRMAN NORQUIST: All those opposed?

[No response.]

CHAIRMAN NORQUIST: Any abstaining? Oh, Barbara is opposed -- okay.

DR. MCNEIL: No. I'm abstaining.

CHAIRMAN NORQUIST: Oh, you're abstaining. Okay. So we have -- oh, you're abstaining, too, or are you just -- you didn't vote? You got to vote one way. You got to make a decision. You can abstain if you want to.

DR. WEISMAN: I'll vote for.

CHAIRMAN NORQUIST: Okay. So Barbara is abstaining, and Ellen. Ellen and Barbara are abstaining, partly because I think they're saying

they don't fully understand what they're voting for. Is that right? Okay. And the others in the room -- Harlan, were you abstaining?

DR. KRUMHOLZ: I was just going to say that, you know, part -- can I just make one comment in the discussion? Part of me, and with people on both sides of me saying --

CHAIRMAN NORQUIST: No, you can't. You can't make a comment in the middle of the vote. We already had our -- you can do it after we have the vote if you want to do that.

DR. KRUMHOLZ: Okay.

CHAIRMAN NORQUIST: Okay. And then I need to hear from Freda.

DR. LEWIS-HALL: Approve.

CHAIRMAN NORQUIST: Okay. Allen.

DR. DOUMA: Approve.

CHAIRMAN NORQUIST: And I don't know if Alicia is on.

[No response.]

CHAIRMAN NORQUIST: Okay. Now did you want to -- so we have -- if you want to make a

comment.

DR. KRUMHOLZ: I just wanted to say one thing because I was sort of thinking about this, again trying to figure out what the board's role is in this. Right? And, you know, I have certain feelings about is this in scope? Out of scope?

Or does this fit in our whole -- but the thing is, in our overriding strategic effort to try to help the CDRNs create a sustainable model, this is a business opportunity for them. I mean, you've got a government agency who's willing to take some of our CDRNs and pay them and, you know, engage them.

And to me, like regardless of what you think about the question and regardless of whether you think this is the right thing, if they're going to be embedded with our principles, which it seems to me they are, and someone external is willing to pay them, like we should put them on the launch pad. I mean, this is a good opportunity for them to drum up business.

So again, I'm trying to think about the

scope.

DR. SELBY: Yeah.

DR. KRUMHOLZ: I know we've already voted, but I'm just --

CHAIRMAN NORQUIST: You know, I'm not just -- I'm also just trying to move on. But I think your point is correct. I mean, it's a small amount of money to put in for an investment. And I think, as Rick Kronick was saying, it's a test of the way we do it versus the way someone else does it.

DR. KRUMHOLZ: Rick, this reminds me. We're paying, but it does seem -- I guess what I'm saying is we're bridging them to the point where they might subsequently have CDC engage them. I don't know. That's --

CHAIRMAN NORQUIST: Well, and we always have the option because of the contract to cut it off if we believe that it's not doing what we want to do.

DR. KRUMHOLZ: Right.

CHAIRMAN NORQUIST: And we haven't even gotten the award yet. We'll have to see. We may

not even get any of this or even make it through the CDC process.

Okay. Lori, you're up, with the -- thanks. So that [inaudible]. Okay. Thank you, Rachael, and the rest of the groups, and the RTC and the SOC, for their work on it.

Okay. Evaluation update.

DR. SELBY: Yes, yes, yes.

CHAIRMAN NORQUIST: So Lori, and we'll take a little bit out of each next presenter's time to give her a little --

DR. SELBY: Yeah. We always get to Lori and Laura and then announce that we're short on time. But I want to reintroduce Lori Frank and Laura Forsythe, who are now part of a renamed sector at PCORI called Evaluation and Analysis.

And it really -- Lori has also been -- or Lori and Laura and others have been riding herd on our data, the data that come out of our various systems for soliciting and reviewing and funding and managing awards. So the data is accumulating. The questions hopefully are accumulating,

particularly questions from you.

And we have an most able group. Behind Lori and Laura stand several analysts who are very good. So -- yeah?

CHAIRMAN NORQUIST: In the interests of time, we're thanking everybody. Let's not list everyone --

DR. SELBY: -- in any way connected to PCORI.

CHAIRMAN NORQUIST: Yeah, yeah. And so -- and for you guys, again, we always want the presentation to be as brief as possible so we can have room for discussion. Okay?

MS. FRANK: Yeah, absolutely. Okay. Thank you, Joe, for that nice introduction. I just want to call attention to the title that Laura has on your first slide there. That's the new title that represents the enhanced responsibilities Laura has agreed to accept for evaluating PCORI. So thank you, Laura.

Okay. To orient us all, we wanted to share some information about what we're learning

about PCORI from some specific evaluation activities. There are two activities that we'll share with you very briefly, and what you're seeing is the evaluation framework. The circled areas are the areas that we'll focus on.

So we'll begin with a discussion about rare disease applications, which is really at that nexus between portfolio management and merit review. And then we'll share a bit about our qualitative findings out of engagement in research activities.

So analysis of rare disease applications: That's here on your map. And this work really began with some questions that had come up around PCORI, including with the rare disease advisory panel, about whether rare disease applications are relatively disadvantaged at PCORI. So we have some data that we could use to examine this question.

There was also an interest in making sure that PCORI maintains a robust portfolio around rare diseases, so you'll see the way to which the findings were used to help support that as well.

So our specific evaluation questions: How many applications on rare diseases do we receive? How many are reviewed at all? How many are sent on to interest in discussion? And then how many are funded compared to applications on other diseases? And compared to those other applications, how likely are they to be discussed, and why, and to be funded, and why?

For today, we're looking at 49 awards that PCORI currently has in the portfolio, just to give you some context for how many awards PCORI has already given. And on this slide we show how they're arrayed across the different funding mechanisms.

So we identified all the research proposals that dealt with rare disease. We're excluding the methods proposals because we had to focus on content in the application around rare disease. We analyzed some data from cycle three; that would be March 2013 through May 2014. That's four cycles there. And as you saw, the questions we had were how many did we receive, and how many

moved on in what ways?

Laura also completed a core comparison. So we can elaborate on some of these data, but just to move through quickly, if you see here, that for this set of cycles, we received 44 applications that had a content focus on rare disease. That represents less than 4 percent of all applications received. And of those funded, nine pertained to rare disease. That's out of a set of 124, and that's about 7 percent.

Laura?

MS. FORSYTHE: Thank you. So as you can see here, we discussed 68 percent of the applications we reviewed on rare diseases. That's shown in the blue bar on the left. And that was significantly more than what we discussed among the applications on other conditions, which is 46 percent, as shown in the green bar there.

Looking just among those applications we discussed, we funded 30 percent of those on rare diseases and 20 percent of those on other conditions. And when we consider all the

applications we reviewed, we ultimately funded 20 percent of those on rare diseases, which again is significantly more than the 9 percent of all other applications that we funded.

We also compared the scores for each criteria in the overall scores. And what we found is that on average, the criteria is overall scores were comparable for applications on rare diseases and other conditions. And notably, they were similar in nature in the scores across all reviewer space, including on impact and technical merit.

And in some cases, the rare disease applications were scored more favorably. Scientist reviewers scored applications on rare diseases more favorably for criterion 5, which is engagement, and patient reviewers scored applications on rare diseases more favorably for criterion 2, the potential to improve healthcare and outcomes, as well as criterion 4, patient-centeredness.

So in summary of this evaluation project, what we learned is that applications on rare diseases are not disadvantaged in supporting their

review. But we do receive a relatively limited number of applications on rare diseases. And we also want to ensure that we continue to address the needs of the rare disease community and attract more applications.

So as a next step, PCORI issued a one-time set-aside funding for rare disease research associated with the spring 2015 cycle for $12 million. And although rare disease applications were not disadvantaged in merit review, these will be scored and considered in separate panels so that we can ensure that experts who know about the conditions and treatments that are being studied are included on those panels.

And we have received 43 letters of intent on rare diseases for that cycle, and 24 of those were invited to submit a full application. And in looking at LOI acceptance rate, 40 -- excuse me, 56 percent of LOIs on rare diseases were accepted compared to 43 percent of all other applications.

And LOIs on rare diseases make up 15 percent of our accepted LOIs for this cycle. And

so that represents a threefold increase, more than threefold increase, in the proportion of the applications that will be on rare diseases.

I also want you to know that we are undergoing an analysis of all of the LOIs we received on rare diseases to look at those we didn't to see if there are any factors that might be unique for rare diseases, like the study design or the demonstration of efficacy that might point us towards accepting LOIs differentially for rare disease groups.

But overall, this work that we've done really reflects our ability to take in a question about our process and find a data-driven answer and then put forward a solution. And we will be evaluating and tracking going forward how this plays out in the applications that we fund.

MS. FRANK: All right. So we're happy to answer any questions.

CHAIRMAN NORQUIST: Larry?

MR. BECKER: This is a clarification question. What's the definition of a rare disease

for this purpose?

MS. FRANK: So affecting 200,000 or fewer individuals.

MR. BECKER: So it would be comparable, identical, to the orphan disease designation.

CHAIRMAN NORQUIST: Rich?

MR. KUNTZ: Just following on that, do we have any -- so 4 percent of the applications is a rare disease and 7 percent of what we funded. Do you have any idea of what percentage of either people or bad things that happened or dollars are -- how is this proportioned relative to burden?

MS. FRANK: That's a really excellent question, and I don't know the answer to that. We can take a look.

DR. DOUMA: This is Allen.

CHAIRMAN NORQUIST: Yes, Allen?

DR. DOUMA: A follow-up to that is I think it's really important to know what we think would be good. So when there's -- and I don't have the slide in front of me, but for example, one slide you talked -- you said we have a limited number of

applications, I believe.

I'm not -- typically, that phraseology means we don't have enough applications. And the question is, what would be enough, and how would we figure that up as compared to, like Rick was saying, the disease burden across all diseases?

MS. FRANK: Right. And that's part of what initiated this work, actually, was questions coming out of the rare disease advisory panel. They're taking a look at all of these questions. What should PCORI be funding in this area?

CHAIRMAN NORQUIST: And isn't one of our -- or two of our CDRNs -- I mean, the PPRNs are rare disease PPRNs?

MS. FRANK: Rare disease across, actually, I believe it's all of the CDRNs can address rare diseases.

CHAIRMAN NORQUIST: Right, right. I'm sorry. I meant the PPRNs.

MS. FRANK: And then on specific PPRNs --

MS. FORSYTHE: Fifty percent of the PPRNs have a rare disease component.

CHAIRMAN NORQUIST: Okay. Other questions about this? Okay. All right. Oh, I'm sorry, Larry. I missed you.

MR. BECKER: Just a point of clarification. This $12 million, is this in addition to other monies that could go to rare diseases, or is this $12 million sort of the circle of money that will be put forward towards rare diseases going forward?

MS. FRANK: Yes. My understanding, it's in addition to. So we can accept applications.

DR. SELBY: Yeah. It's a set-aside within a specified overall amount of funding. If rare diseases did so well that they collected all the set-aside of 12 million but there were others that scored highly, we may wind up funding even a higher fraction than the set-aside.

DR. WEISMAN: Can you -- I mean, I'm all for this. But can you explain the need and why the need for the set-aside since it appears they're doing well. We should have the other ones doing as well as they're doing.

MS. FORSYTHE: So the set-aside --

DR. WEISMAN: We should set aside for the others.

MS. FORSYTHE: Well, so, you know, the issue we identified was that it's not that we're getting a bunch of applications on rare diseases, and then because they have a different lens and you look at impact or technical merit, we're not funding them.

It was that we were getting too few applications from the perspective of our rare disease advisory panel. And one of the issues they told us about was that it's not clear to the rare disease community that this is a priority for PCORI and that they fit for our research priorities and what they're looking for.

And so this is something identified as one way to communicate that we value these applications and are looking to study rare diseases.

DR. WEISMAN: Yeah. We want to give them a bigger piece of the pie even if they're funded at a high percentage.

MS. FORSYTHE: Right.

DR. WEISMAN: We want to do more funding--

MS. FRANK: That's the reason --

CHAIRMAN NORQUIST: I mean, I think we want to be clear publicly, yes. Rare disease is important.

DR. WEISMAN: Aren't we -- that's the part I'm missing. So --

CHAIRMAN NORQUIST: What's missed?

DR. WEISMAN: Well, we're funding --

CHAIRMAN NORQUIST: Yeah, yeah.

DR. WEISMAN: We're not going to change our review criteria.

CHAIRMAN NORQUIST: No.

DR. WEISMAN: So if they -- which means that their applications are a very high quality. And since we tend to fund them if we get them, is throwing more money at it the key or is soliciting more applications the key? Because if we -- the problem is not, it seems to me, not that we're not -- once we get them in, that we don't fund them.

The problem is we're not getting enough

applications because if we got the applications and you projected the funding rate, we would be funding even more.

CHAIRMAN NORQUIST: Right. So you want to answer that?

MS. FRANK: Remember where this started was we didn't even know the answer to that first question. So were they disadvantaged in any way? So now we can say no. In fact, they appear not to be relatively disadvantaged. Had they been, we would be discussing a set of action steps to address that.

And then, just as we concluded here, in the absence of a specific metric, the answer is rare disease advisory panel and PCORI want to fund more, which was the reason for the set-aside.

DR. WEISMAN: But now that we know the answer, maybe the right response to setting aside more money is finding out a way of how we more effectively get applications in to us. That's --

MS. FRANK: Yeah, absolutely. So outreach, actually, to these are part of the

answer. Absolutely.

CHAIRMAN NORQUIST: Bob Zwolak? Larry, is yours up, or is that from before? Yeah. Bob?

DR. ZWOLAK: I mean, I favor this concept because I compare it sort of to the large pragmatic trials. And we have set-asides for large pragmatic trials, and it's unlikely that a rare disease would be a large pragmatic trial. So I think it's a great idea.

CHAIRMAN NORQUIST: Okay. Other questions about this?

[No response.]

CHAIRMAN NORQUIST: Okay. Are there other topics you're going to talk about?

MS. FRANK: We do have more to share out of evaluations. We can make it quick.

CHAIRMAN NORQUIST: Yeah. If you could make it -- well, I mean, how much more do you have to -- yeah.

MS. FRANK: Joe says let's go ahead. Okay. So this part is about understanding engagement in research. This is your "you are

here" map.

CHAIRMAN NORQUIST: Yes. We want to hear this because we had a discussion earlier.

MS. FRANK: Yes. Exactly. So what we wanted to do was to measure the ways in which engagement affects or impacts PCORI's strategic goals. So on that conceptual framework, we can see increasing useful information and getting speedier uptake of that information. And so part of what we're trying to do is to learn about engagement in PCORI research projects so that we can understand what impact there might be specifically on those strategic goals.

We shared with you in December some quantitative data out of our examination of engagement in research among awardees, our purposes are still these. We want to describe what's happening first and foremost. Evaluate that impact on PCORI's strategic goals, as I mentioned.

We want to be sure to feed back what we learned into the PCORI application guidelines. We want to guide awardees and the field; and then

finally, support projects, support all awardees in their projects, and so, obviously, working with everyone around PCORI engagement and science, especially.

So we have the Ways of Engaging-ENgagement ACtivity Tool, which we call the WE-ENACT. And we will be sharing with you some qualitative data -- I'll set some context -- but some quantitative data to begin with.

Just a note that we asked the researchers to nominate their research partners or stakeholder partners to also respond to our set of questions. And here are the data that we have as of now, so on the left, 186 responses from researchers and 299 from patient or stakeholder partners.

We partnered with the American Institutes for Research on a qualitative analysis with the open-ended text that we received back from our questions. And I specifically want to acknowledge Tom Workman and the team at AIR. So that's Maureen Maurer, Emily Alfstad, Deepa Ganachari, and Marla Clayman.

So with them, we developed and applied a code book. We had a set of research questions that guided that part of the work. And we reviewed these open text responses in great detail. So it was an iterative process, as a qualitative analysis often is. We identified major themes that emerged, and then matched those teams to our conceptual model of patient-centered outcomes research. So that was really used as our guide for the analyses.

So those qualitative research questions are these: What strategies are being used in engagement among awardees? What are the barriers and facilitators that they identify? What's the impact of engagement, even at these relatively early stages? Remember, we're dealing with baseline and year one data. And are there any differences by patients or patient or stakeholder partners? And what can we learn about improving the way in which we're collecting information from everyone?

So we analyzed these data using content codes. And the point I want to make here is that

we're avoiding anecdotes. So there needed to be 25 or more responses in order for a code to rise to the level of a theme. But these are the themes that emerged.

It was around engagement strategies and impact barriers and facilitators, the way to which the partnership emerged and is maintained. We had a lot of themes emerge around training and logistical issues, and then PCOR principles, which addresses elements in the engagement rubric. So that was an interesting first step towards understanding and validating that for us.

I'm showing you the simplified version of the conceptual model of patient-centered outcomes research because this [inaudible] our analyses -- excuse me. And this is how we will explain these qualitative findings. So it's basically structure, process, and outcomes. And we'll focus in on that center column, the process or the action items there.

And in terms of setting context with the quantitative results: So here are many of those

different approaches to engagement that the awardees have been reporting to us. The convention is when you see blue, it means it's researcher report. When you see red, it's patient or stakeholder report. So these are researcher reports. You can see advisory group as a common approach used to engagement. These categories are not mutually exclusive.

And we asked, when in the process of research are you engaging with patients or other stakeholders? And the answer is, across all stages. You can see here certainly identifying research topics and developing the research questions, a prominent stage for engagement. But all throughout, it's quite gratifying to see the extent to which engagement is being practiced across the life cycle of a project.

And then we said, who? Who are these stakeholders that you're engaging? So at the top, if you click "patients and clinicians," this is an action item that emerges from looking at the data. We can see that we could focus more on engagement

of other types of stakeholders in PCORI-funded projects. So we can see payers and purchasers, for example.

MS. FORSYTHE: As we move into sharing some of the qualitative findings, I want to remind you that we're really going to be looking at those themes that we chose based on their relative frequency and their robust -- their presence across the data. And we're going to start with looking at initiating and maintaining partnerships.

And one thing that came through very clearly was about both the benefits and challenges of early engagement as a way to foster strong relationships. Patients and stakeholders who were involved early really noted how useful that was, and many who weren't involved early expressed the desire to have been involved earlier. As you can by this patient stakeholder quote, "I wish they would have contacted us earlier so we would have been able to work in more areas of the state versus a small section."

But researchers really noted a lot of

challenges related to early engagement. They noted it's difficult to keep patients engaged throughout the project, and in particular, it's difficult to set expectations for project funding.

The quote on the right from a researcher here talks about how going back to stakeholders when a project isn't funded is really difficult, and in their view, this is the greatest challenge of involving patients in conceptualizing and planning a research project.

We also heard about financial challenges, in particular, lacking the funds before you get an award from PCORI to collaborate on the proposal, and also other logistical things like institutions requiring that you take consultants off of an existing grant or contract line.

And so one thing I want to note about these challenges that researchers highlighted is that they point towards a transitional more than a relational view of engagement. And this highlights an opportunity for PCORI to try to shift the thinking towards engagement in a program of

research rather than in a singular study, but that this is still a challenge for researchers. And also, we are going to work with our engagement team to try to understand better some of these issues around setting expectations and the extent to which that's a problem for both partners and researchers.

We're going to move to talking about communication now, and several themes emerged. One that is notable was the importance of creating an open environment for sharing. And we heard a lot about the importance of managing power differentials, and in particular, with patients and clinicians working on the same project.

So this quote on the left here from a researcher talks about, "Still working on whether stakeholders should meet together or whether patients might not want that because it can be intimidating." And, "Asking questions about how relationships and conversations can be facilitated when different groups feel very strongly about issues in different directions."

We also heard a lot about managing diverse

groups that require cultural sensitivity. And this stakeholder highlighted the importance of researchers needing to understand how to communicate with people who are not of the same age or cultural background. And both patients and stakeholders and researchers alike highlighted the importance of using plain language and really speaking the same language to each other so that everyone can participate.

And so we really learned a wealth of information about the variety of ways that researchers and their teams are capturing and using the perspective of their partner. We're going to focus today on study design and recruitment and retention of important steps along the way, and capturing of patient perspective.

So for study design, both researchers and patients and stakeholders highlighted involvement of partners in choosing the patient groups to study, and identifying measures and interventions and the appropriate comparators and the outcomes to measure.

There's several examples here. In this case, giving clinical input on the screening measures was about the timing for when one would decide that a mammogram or a Pap smear was overdue. And I think this is a really interesting quote about working with stakeholders to decide what is the appropriate control group for their clinical trial. Is it standard practice or is it some other currently existing intervention that may be ineffective?

We also heard about partners working together to decide the best way to collect data, and also reviewing and revising study plans and materials that are handed out as part of interventions.

In talking about recruitment and retention, we wanted to bring this to your attention because of the volume of responses, particularly from patients and stakeholders about this being an important way they were involved.

We heard a lot about strategizing for recruitment and retention, and partners helping to

bring the patient perspective to this element, and figuring out why might someone choose to participate in a study, and how should they be appropriately compensated, for example.

So as this stakeholder quote says, "We helped them understand potential barriers to enrollment, particularly for minority candidates, and identified responses to these barriers." And partners also served as liaisons between the research team and the groups to be recruited from. And also, we heard a number of comments about on-the-ground recruiting, handing our flyers, doing screening and reviews, and speaking with people about their eligibility for the study.

And so now we'll move on to ensuring influence. This is where we stop and examine what is the early impact on the study itself. And we'll look again at study design and recruitment and retention.

The researchers were divided in their views on the impact of engagement on study design. Many said there was really a large impact on the

study design, but some said that the impact was more minimal or that the patients' element in designing the study design seemed unnecessary.

But here are some of the examples of things that do highlight when folks felt that it was impactful in terms of making the study more responsive to patient needs and more feasible in the clinical setting.

This patient or stakeholder talked about "contributing to approach that really allowed for maximum participation of both patients and providers"; a researcher talking about adding a third study arm; and others talking about changing the design and the flow of the study or the timeline of the study assessment based on their partner input.

Both patients and stakeholders and researchers identified a number of ways in which recruitment and retention were affected by the partnerships, in particular pointing towards recruitment procedures being more responsive to patient needs; also, changing recruitment messages,

getting more people aware of the study, and really helping with recruiting and retaining difficult-to-reach populations.

And so this quote on the left here is notable. "Since discussing our challenges with recruitment with our participants, we've only had one person decline to participate."

We also want to spend a moment talking with you about training. Both the partners and the researchers really emphasized the importance of training, and often talked about having to go and seek out this training on their own. Researchers really highlighted the quick and steep learning curve for patients and stakeholders to understand a lot of information when they come onto a project.

Some of the topics that our respondents identified for which they need more help with training are on the topic background that they're studying and research methods, but also some issues that are most cross-cutting, like how to help train stakeholders in how to provide input effectively so to feel like an equal voice at the table, and how

to put in context their personal experience to help inform the study.

And then also, how to effectively communicate back the findings from the study to the communities that they represent. And researchers also are really asking for our help about how to partner.

And I just want to remind you that again, these data are very early. We were hearing from people either at baseline or one year into their study. But nonetheless, we asked them about early outcomes beyond the study, and we heard a few important themes.

First we heard from partners in particular about increased knowledge and skills about research, and that they're learning things that they are going to apply in other settings, but not just professional and research settings. Also, some partners talked about learning things about engagement in research that they can now translate into their own healthcare and be more involved in their care.

Additionally, we heard from both groups about increased interest in working with patients and stakeholders in research projects. So this researcher is telling us, now I feel more comfortable, suggesting that we include patients on our projects.

And on the right here, one of our patient or stakeholder respondents talked about their groups more formalizing their connection to researchers and establishing ways that they can reach out and find researchers to work with them on other projects that address their population of interest.

And finally, a variety of stakeholders talked about other impacts beyond the project, like feeling they have more influence in their community, that they're affecting health policy, and other aspects of care through their work on the research projects.

And the last thing I want to note from these qualitative data is that we built in the PCOR principles that Lori mentioned coming right out of

the engagement rubric as the ethical backdrop of our conceptual model of PCOR.

And we felt like this qualitative analysis really provided evidence for these principles in the lived experience of our projects. They were woven throughout everything that we read about, and in particular, the things that we highlighted today, for example related to training and compensation and early engagement.

MS. FRANK: Okay. Thanks, Laura. So that was a lot of qualitative data. So you can see this is just one example of the ways in which we are learning about engagement directly from our awardees and then turning that learning back into useful action.

So some of the top lines out of this: It's challenging for researchers to develop these partnerships with uncertainty about funding. So the viability of the partnership was presented, especially in the qualitative findings, as a challenge.

As Laura noted, the impacts on study

design ranged anywhere from quite minimal to quite dramatic. Recruitment methods really emerged as an important area for the value of engagements. That's an area, obviously, we want to follow up on in different ways.

Respondents noted their training needs. And as I mentioned, Sue Sheridan and partners are already working on helping to turn this information into full training for the communities.

And we have early evidence, even out of this early stage of the process, about the impact of engagement, and we'll be continuing to follow up so that we can understand really what difference this PCORI requirement makes.

On that point about challenges at all stages and how the awardees have been dealing with those challenges, it's really important for us to turn what we're learning back into improvement for PCORI and for awardees.

So we have some real points out of this qualitative analysis, supported by the quantitative analysis as well, results of which you've seen

before -- the setting expectations as to applications phase, what success probability is.

Different types of managing a research relationship: So we heard a lot about the value of face time, but also technology solutions for dealing with remote teams. Protected time for researchers to help with establishing and maintaining partnerships.

We see the opportunities to expand stakeholder engagement into some of those groups, where we see evidence of less engagement in our own portfolio. Training, as I've mentioned. And then themes sort of share what we're learning with the wider community. So this is just one example.

The methods program has an evidence-to-action network, so we were able to share these results with them. It included the researchers and the patient and stakeholder partners, and we had a really interesting discussion with panelists out of the awardees.

And these particular data help us to establish an inventory of engagement activities,

which will give us essentially a vocabulary for understanding engagement moving forward. And it's our first step towards, as we discussed, validating the rubric.

So we're always glad to share what we're doing in terms of evaluating PCORI, and look forward to your questions.

CHAIRMAN NORQUIST: Questions? Bob? Are you going raise the --

DR. ZWOLAK: That was quick.

CHAIRMAN NORQUIST: Yeah, yeah. I'm watching. Yeah. Wait just one minute. Bob Zwolak, and then Allen, we'll let you go.

DR. ZWOLAK: So that's very nice, I think very helpful. But I've missed one thing. These were all awardees. Did you assess any or send any questionnaires or seek data from people who had their grant applications rejected?

MS. FRANK: We're not asking folks who aren't funded by PCORI about engagement in their research. This is really a focus on engagement. But we are -- we do have a researcher survey that

includes individuals who have and who have not been funded by PCORI.

DR. ZWOLAK: But it's only to say that it seems like failure of engagement or poor engagement may have been the cause by some people who weren't awarded grants.

MS. FRANK: Yes. That's an interesting point. I don't know that we could come to that conclusion, especially not out of these data. We always make the point that we have other analyses, including some merit score analyses that Laura's leading, and technical merit is prime.

So applications must meet a certain standard for methodologic quality. And then obviously we have the reviewers comment on a range of other aspects, including the engagement. Part of what's interesting here is we know what they say at the application phase with what's really happening once they're out there conducting the research.

CHAIRMAN NORQUIST: So that will be another key part in evaluation, is what happens

after the award. And plus you do have an internal opportunity because you'd have people who resubmit. So you have people who get rejected the first time but then resubmit who you then would survey, and you might find out something about what happened the first time. Right?

MS. FRANK: Right. What changed.

CHAIRMAN NORQUIST: So there's that opportunity. But there's also the opportunity for some -- we do need some evaluation of what's happening after these projects are funded.

MS. FRANK: Right. So these actually are folks, some of them as long as one year into the process.

CHAIRMAN NORQUIST: Okay. Yeah. So Allen?

DR. DOUMA: Yes. I just want to comment. I think this is a great way to create a catalogue of all the various components or issues that we or PCORI is dealing with on a day-to-day basis with regard to engagement.

But on the flip side of that is -- and

just my overview in this short time we've had so far -- there's not -- I don't see anything that's really surprising, I wouldn't have suspected these as issues already.

Apropos to that, were there any unexpected findings? And if so, is there anything in particular that we can do to focus on those?

MS. FRANK: Yeah. So that's a wonderful question. So I mentioned that one of our early goals was to describe engagement. We all had ideas going in about how things would look. So I was surprised, honestly, by the ways in which our early impressions or our early hypotheses are concerned. This is what's happening with the engagement.

Honestly, I was surprised to see such a range of approaches to engagement, multiple types of engagement approaches being used within the same project. So it's not just that awardees essentially focus on one form of engagement and then propagated that through the rest of their work. They are active in multiple ways.

I've heard a lot from the discussions

particularly of the challenges and how teams have overcome them. I think it's very helpful for future awardees, but for PCORI in terms of our application guidelines and ways in which we already have clarified some points in response to that [inaudible].

CHAIRMAN NORQUIST: Okay. Rick and then Christine.

MR. KRONICK: This was great. Thank you, Lori and Laura. And this will be a broken record here. As you do this work, the pressing need that we I think all have to respond to this "So what?" question about engagement is one that I'm sure you're paying attention to. But to the extent that you can help us with that, it would be great.

I mean, I saw one little blurb on "So what?" There's a third arm in a trial that would have otherwise have two arms. That's helpful. It's not, you know, really sexy and exciting, but it's helpful. But any other stories of what is different, as you pointed out, that recruitment is helped.

On the other hand, we saw on the dashboard earlier that, you know, recruitment milestones are not being met so well. I don't know how that compares to recruiting milestones of NIH trials or our trials when we used to do trials. I'm not so sure it's different.

So a broken record, but you at least continue pushing for more --

MS. FRANK: Yeah. And thanks for raising that. Absolutely. So part of it is the near term outcomes are always addressed right now. But we --

MR. KRONICK: Even on near term, to be able to say that, you know, a question, if it's asked differently, or question that's asked that wouldn't otherwise have been asked, or that the study was designed differently in some, you know, way that's much more likely to make it successful -- you know, something that is [inaudible].

CHAIRMAN NORQUIST: Okay. Christine?

MS. GOERTZ: Thank you both. This is really an excellent presentation, and to everyone who actually has been working on this project.

This makes me think a little bit about some of the discussion we had earlier about what are the barriers to the submission of application. And I'm just wondering to what extent we've looked at engagement as a barrier.

I know that you have a little bit, and I know that we're able to look at merit review scores and see the extent to which the engagement portion of the application has been a barrier to funding. I'm wondering if we might want to look at that in a little bit more detail, and then also consider the possibility of providing a small amount of funding to potential investigators in order to enhance engagement.

I mean, a couple thousand dollars could really go a long way towards funding those efforts. And also the possibility of sharing successful engagement plans online, you know, both patient-centeredness and engagement plans online, so that people would be asking investigators if they would be willing to share that so that others are able to use those as models and get a better idea of what

this really means.

So that's one of the things that I hear out there. I don't really know what you mean by patient engagement. And so, anyway, just a couple of suggestions.

MS. FRANK: Yeah. Thank you.

CHAIRMAN NORQUIST: I think that's good. I think the other thing is it's not always the money. You need sometimes someone to help you understand better to engage. Having done a lot of this, people just fundamentally don't understand. They need a mentor in some sense.

And that may be one thing to think about, is how you get a -- now that you're learning all this and there are some people who are good at it, is partnering them with people that might be helpful to get them off the ground.

Harlan Krumholz?

DR. KRUMHOLZ: I just want to congratulate you on an incredibly thorough approach, and to get back to this thing I've been talking to Robin about, which is, how do we move this stuff into

education? And, you know, I'm really impatient about this.

I'm feeling very -- I wanted to be able to start a course this fall based on materials that are produced. They aren't produced yet. I'm seeing this, and I'm going, like this is just great stuff to funnel into course work material, whether it's the move or something else.

I mean, this should be education for researchers, and there ought to be ways to structure primary data into ways of teaching and bringing together this with literature so it's both -- you know, we're reporting back what our experience is, but we're also putting in the context of what's been published and what's out there regarding issues around engagement.

And it just seems so prime to me for people who -- I mean, I would love to take -- I have a PCOR, 12, and I would love to take every one of my 12 scholars and have them go through a course where they're learning about these things in a course that PCORI has been -- you know, has

[inaudible] to create.

So maybe with AHRQ, there's some ways to be able to bridge this. But, you know, I'm looking and listening, and I'm just thinking like that there are many places around the country, if they want to embrace this way of doing work, they need exposure to things you're learning, and also in the context of, you know, a framework that's been not just what we're developing but what others have done.

So I just want to urge us to think about how we can redouble effort to get out this educational material, both for classes, for all the curricula. I just don't think we're doing enough. And even those contracts that have been let, I wish that we could invest more, and with AHRQ, in trying to get this done.

But, I mean, I'm just saying this seems to me to be the -- would be very fertile for education. And so I would like to pursue that.

CHAIRMAN NORQUIST: Yeah. I think that's a good point, and that was my point to Christine,

too, because I think if you're going to help people do this, you've got to educate them. And I think I would just add, it's not just for teaching at the institutions.

It's also to teach our other stakeholders and those that have an interest in us because when we go up on the Hill and others, I mean, it would be very nice if we had these messages very concrete and that we could work on.

Let me go to Sharon first and we'll come back. Oh, did you have a follow-up on this particular --

MR. KRONICK: Just that we do, at AHRQ, lots of tool development and education. And this would be ripe for that in some ways. But the submissions part is the evidence about what using these tools will do.

CHAIRMAN NORQUIST: Right. The content. Yeah, yeah.

MR. KRONICK: And so I'd be uncomfortable with pushing this very far.

CHAIRMAN NORQUIST: Right. Right. Okay.

Sharon?

DR. LEVINE: Yes. Rick, I wanted to respond to your early question about the "So what?" And I think the ultimate measure of "So what?" is the speed of dissemination and the speed of uptake from research results when the process has been heavily -- has an early and heavy investment in engagement. And to some extent, that is a comparative effectiveness study.

CHAIRMAN NORQUIST: I mean, if you can change the timeline of moving something into actual use, that would be a huge outcome. Right? You're absolutely right, yeah. We won't know that for a while.

Okay. Any other comments?

[No response.]

CHAIRMAN NORQUIST: Thank you both, Lori and Laura, for a very wonderful presentation and the work here doing. I'm sorry. Leah, did you have a --

MS. HOLE-MARSHALL: I just had a quick clarification, and it was related to the committees

over here. When you talked about training in here, is that training that you all are developing, or that's an area where you are asking about and know that people want? Like something said, I was searching the web and I couldn't find anything.

MS. FRANK: Yeah. So it's both. And Sue and team are working on that.

MS. HOLE-MARSHALL: Great.

CHAIRMAN NORQUIST: Okay. Thank you very much. Thanks.

DR. LEWIS-HALL: Yes. I'm sorry, I wasn't sure our place to jump in.

CHAIRMAN NORQUIST: Oh, Freda?

DR. LEWIS-HALL: I just had two quick points. One was that asking investigators is not always, you know, a good way to find out what would have been different because these changes are often evolutionary within even the context of a protocol. So it's kind of a soft turn, if you would.

The other question that I want to ask when you're looking for examples of what a difference was made is to look for some failures, so to look

for examples where patient input was not received, and at the end of the day, the study missed its mark in some important way.

So just a little bit of a different way; to get positive examples might be harder than to get negative ones. And then you can use the negative ones to support change because, you know, change happens in [inaudible].

And then on the education, it wasn't clear to me who was actually developing the content. I think you were talking about it, and you faded a little bit, and then I just couldn't exactly hear who it was.

MS. FRANK: Yeah. So this conversation is with the engagement team, and Sue Sheridan in particular, who is very close to these particular learnings to feed that back into a curriculum of some sort. And so I'll let Sue address future questions on that.

With regard to your other points, though, I think that the respondents has been very generous about sharing their successes and their failures.

And we're learning, even from this group, from both. But we are looking for comparative ways to understand the "So what?" question. What difference does it make? Compared to what?

CHAIRMAN NORQUIST: Thank you. Again, I'd thank you. And then one thing that we did mention this morning at some point, we would like to hear what you guys are learning about successes and failures of people applying for pragmatic clinical trials.

Okay? Thanks.

So we will now take a break, and we will be back at 3:35 Eastern Daylight time, so 35 minutes after the hour.

[Recess.]

CHAIRMAN NORQUIST: We're back.

DR. LEWIS-HALL: Freda is on the line.

DR. DOUMA: Allen is on the line.

CHAIRMAN NORQUIST: Steve Goodman is also on the line, I think.

DR. GOODMAN: Yes, I am.

CHAIRMAN NORQUIST: Robin?

DR. NEWHOUSE: Hi, everybody. I'm pleased to update you on the activities of the Methodology Committee, but before we go too far, I want to make sure I introduce Dr. Cynthia Girman, who has just joined us in September. She has absolutely become heavily engaged in the Methodology Committee work.

Cynthia is from Merck and now is a consultant. She retired last year, and now has an active consulting business. She's already become involved in some methods related to patient-reported outcomes, data management, the charter, and many other things. We are so pleased to have Cynthia with us, and we are glad you are here.

Today, first of all, we have gone through a process by which we were setting our strategic priorities, but we will first give you an overview of the types of activities we have completed and have planned.

Second, update you on a generation of new methodology standards and a review of our current methodology standards. There are two in development. One is design with clusters, and one

is standards on complex interventions.

Next, we will discuss some of the methods, the apps, that have emerged, and our activities to actually address some of those methods gaps in a couple of different areas. First of all, in the area of PCORnet, second, usual care, a third, a deep dive around health care decision science, and a fourth, workshop around patient-reported outcomes.

Then we will move on and discuss some dissemination of the methodology standards and the augmentation of the training, both in terms of CME and academic curriculum.

Then we have two updates. I'm not sure if you got the e-mail, but we received word that we have a new Methodology Committee member. We are delighted. I'll save that as a surprise.

Let's get started. Steve and Cindy, please feel free to chime in or add to this presentation as well.

First, since the last meeting, we have engaged in a priority setting activity, and these

are three areas, first of all, that we are focusing on generation of methodology standards, second, the methods gaps, and third, dissemination of standards.

As I said, I'll tell you a little more about the design with clusters and the complex interventions, and review of the methodology standards. I'm going to go on and not say much more here.

First of all, in terms of creating standards for designs with clusters, we took a little different approach in this generation activity in that there are a number of experts that are already in the field writing and thinking about how to approach issues on designs that use clusters.

In this case, we assembled a group of experts that came together, Dr. Kopsoll [phonetic], Simon, Murray, and Donner, that met with us on April 7. We developed a list of straw man standards based on the evidence, and then came together in a workshop led by David Hickam, and the

experts dialogued and debated on the standards around designs with clusters.

We had a very fruitful day. On Wednesday, the Methodology Committee will be reviewing those standards and making a determination if they are ready and mature to move forward.

The Methodology Committee members that are involved in this activity include Naomi Aronson, Cynthia Girman, Robert Kaplan, Sally Morton, myself, and Sebastian Schneeweiss.

The second standard generation activity is around complex interventions, in this case, we also are taking a little different approach than we did in the development of the first set of standards. We have created a work group of Methodology Committee members. Brian Mittman is leading this activity with Naomi Aronson, Dave Flum, myself, and Mary Tinetti. This work group has two major steps. First is around definitions, and the second is around identifying the current guidance statements that are already available.

The first step is around the literature,

around the scope and definitions, second, what guidance is already out there. Brian will convene this work group and will recommend standards that will be deliberated in the fall.

MS. HOLE-MARSHALL: I just have a quick clarifying question. When you say "complex interventions," that means something that includes multiple components? Can you say a word about what that is?

DR. NEWHOUSE: Yes. When I think about it, many of your health system interventions have complex parts, trying to identify what the active components are of the intervention and how to measure them well.

It's hard to believe that in 2012 we presented the first set of methodology standards, and here we are in 2015 and we are now in the process of reviewing the current standards. We have completed the review of four sets, first, formulating research questions. The second associated with patient-centeredness, and now we are working on heterogeneity of treatment effect

and causal inference methods.

Our intent is by the fall, we would have reviewed all of the standards. The PCORI staff have been incredibly helpful in this review because they have identified areas where they have had additional questions related to how the standards would be used in application.

It has helped to clarify our language. Although at this point, there haven't been any large substantial changes, but there have been clarification changes made.

The next step is to complete the seven categories over the next five months and have them completed by the end of the year and revised.

In terms of prioritizing methods development opportunities for PCORnet, we have had a very active group. As I mentioned earlier, the Methodology Committee has a number of members that have been involved in PCORnet activities, so the methods issues naturally emerged.

Sebastian Schneeweiss has very early been working on distributive data network, and Sally

Morton identified needs around missing data. Cynthia, Steve, Sally, and Sebastian have come together to formalize that relationship. We had a meeting with Rachel two weeks ago to try to formalize that relationship to make sure we could be helpful. I should add that Ethan Basch has also been involved in the patient-reported outcomes work as well.

Another initiative is being led by Hal Sox. He presented some issues around the definitions of usual care, and we are putting together a work group - Naomi Aronson, Ethan Basch, Mark Helfand, David Meltzer, Neil Powe, and Mary Tinetti -- to make a clear statement about the definition of "usual care" and the implications to PCORI.

Another activity that we have been involved in is a deep dive in health care decision science. This work actually started last year, led by Dave Flum, Mark Helfand, and Dave Meltzer. They have worked to develop a plan for a workshop to explore issues in decision science. The plan is to

work with contractors to develop a workshop and hold that workshop later in the fall.

The next initiative is a workshop around patient-reported outcomes in electronic health records. That work is being led by Ethan Basch in collaboration with Lori. The idea is that the work that was completed in the patient-reported outcomes in November of 2013, the workshop identified the state of the science in patient-reported outcomes, and now Ethan would like to take that work forward in a workshop with Lori Frank to help to understand the implications for using patient-reported outcomes in an electronic medical record.

The members of that work group are Naomi Aronson, David Flum, Cynthia Girman, Bob Kaplan, Neil Powe, and Mary Tinetti.

In terms of training for methodology standards, the need for training materials both in terms of CE, continuing education, and CME, as well as the academic curriculum was pretty clear, the recommendations for both of those. Training activities occurred last year, and the PCORI staff

has been absolutely wonderful in moving it forward.

The first bullet is to discuss Baylor College of Medicine to receive a contract for CME/CE methodology standards training. It is underway with a deliverable expected at the end of spring, in May. Mark Helfand is involved in that activity, and a number of Methodology Committee members and outside experts have been invited to assist with that curriculum development.

The second is the academic curriculum that Harlan mentioned a little bit earlier. That contract has been awarded to Johns Hopkins. The contact for PCORI staff is working with the contractors in developing the academic curriculum, which will be expected in September of 2015.

In conclusion, we were just notified that we have a new Methodology Committee member. We'd like to welcome Adam Wilcox. Adam is Medical Informatics Director at Intermountain Healthcare. He holds a Bachelor's in Physics and Mathematics from the University of Utah, an M.S. in Medical Informatics, and a Ph.D. in Medical Informatics

from Columbia University.

Based on the discussion this morning about the implications for medical informatics, we are delighted to have an expert join our team, and we welcome Adam to the Methodology Committee.

I will close, and on behalf of the Methodology Committee, open the floor for questions.

CHAIRMAN NORQUIST: Thanks, Robin. Steve?

DR. GOODMAN: I don't have anything to add right now. I'll be happy to help answer questions if I'm needed.

CHAIRMAN NORQUIST: Thanks. Joe?

DR. SHELBY: A couple of things. One, first of all, it is so exciting. This is a slew of activities, and it is very exciting, Robin and Steve, to see how much activity is going on, the notion of new standards and revised standards is exciting, but also to do questions like the usual care questions.

Usual care, that has been an issue that comes up. Barbara and I just had an exchange about

it. It comes up in critiquing what we fund. Would it make sense to add some SOC members to that right off the bat? That's one question.

The other question had to do with Harlan's request. I wondered if you could distinguish those two curriculum development projects, the one with Baylor, the one with Hopkins, and answer whether either one of those might be what Harlan has been looking for.

DR. NEWHOUSE: The CME activity is around training activities for clinicians essentially. That work is developing a number of modules that will be provided free of charge for PCORI, essentially for clinicians to understand the methodology standards.

The academic curriculum is more targeted toward graduate study, so the types of students that we see in the academic environment and the course work required and the learning activities focused on the academic curriculum. So, different populations.

DR. KRUMHOLZ: Part of the back and forth

we had this week was because I thought we had been really pushing this forward quickly, and of course, I'm always a little impatient about it, but can we just be clear what the product is going to be from Hopkins? What will they deliver?

DR. NEWHOUSE: In the interchange, I think we have the opportunity to intersect and work with them on the development of what the product is, but it is academic curriculum that we could use. I know there was some interchange about the possibility of having publicly available media that is web accessible. It is around modules for academic curriculum.

DR. KRUMHOLZ: The scope of work has been let. We should know what is deliverable.

CHAIRMAN NORQUIST: Does anybody have an idea of what was in the deliverable of the contract?

DR. KRUMHOLZ: Just on a broader point, I know we are working with AHRQ on the dissemination, but it seems to me on the education side, we could be putting it out for multiple audiences. What I

am looking for is can someone hand me something that can turn into a course that could be a foundation for a course.

Have you seen these things where they are teaching people where they are drawing things on blackboards? It could be information that could be assigned to people that could get them up to speed, whether they are working in state Medicaid offices or whether they are trying to -- no matter where they are, it just seems to me there is an abundance of places where there is a lot of fuzziness around what the content is here.

We have a chance to sort of form the foundation of what PCOR is, even the whole notion of PCOR. Again, we have so many balls in the air. Joe, this is just meant to be constructive. I know just keeping this ship together is a big thing.

If it's about resources, I hope we can assign enough resources to create this concept. It just seems to me like it's our chance, part of our legacy, to really codify a lot of this stuff. These things will evolve, by the way, as science

emerges, they will continue to evolve. Between the talent we have in the Methodology Committee, the products you have already created, the products I see in front of us, that funneling into ways of educating people, both chunk, courses, various different levels, coordinating with AHRQ.

It just seems to me to be an immense opportunity that we should be sprinting toward. In patient training and all these things, people are confused about what these terms are. We are the YouTube that people go to on comparative effectiveness. I love those things on the blackboard. They are kind of telling a story. It's edgy and cool and it's also full of content.

We should be thinking of ways to leverage this in advance ways. You get my drift about it. I just want to sort of reenergize our effort to really pull forth all the good work that the MC has been doing and get it out there in teaching material.

DR. NEWHOUSE: Thank you. I certainly appreciate there is an opportunity for intersection

from the Board and from the Methodology Committee. It is a staff activity, but we certainly can provide lots of input.

DR. KRUMHOLZ: Yes. I think our role again, if we fully endorse this and say we are willing to put resources behind it, of course, we are charging Joe to implement it, that's our job to say is this strategically important to us, and we want to be updated on the progress, the operational progress, but we are not going to micromanage it.

I think it is fair for the Board to ask what exactly is the deliverable of the contracts, do we have an idea of whether or not we are advancing -- I don't need to see the whole contract, what is going to happen when.

The other thing finally -- I know you guys want me to shut up, but this is the last thing, it's public stuff. Then we can sign in on Twitter, yeah, please have him shut up.

CHAIRMAN NORQUIST: I'm not looking at Twitter.

DR. KRUMHOLZ: If I knew there was going

to be material that could be the basis of a course, one thing I might want to be doing right now is alert teachers around the country so that people could start thinking about okay, if I wanted to do a course, maybe I would do one next spring.

I will tell you, I, myself, have been thinking I'd love to do a course in this, and I have been waiting for baited breath for this. I could do my own curriculum, but I was hoping this would help me. I was going to do it this spring. I was waiting. Then I was going to do it in the fall. I have been telling people I was going to do it in the fall. Now I'm just going to have to do it because it doesn't seem like it's going to come in time, and that's fine.

You would want to let teachers around the country and in every place, in chiropractic schools, and all around the country, to let them know this is going to be ready for you. If you're interested in trying to have a course, you're going to get a lot of help. It's going to come when, you know. To plan a course, you have to let people

know in advance when the course is going to be.

Even if you could come out with what the course objectives might be that we're going to fill in, it helps people know as they develop their rubric, their curricula, their reading list, all these things are going to be part and parcel of getting help.

If you can produce even some chunks so we can assign people, hey, we have created some content that you assign to the students in addition to their reading, so when they come to the classroom, they are ready to be taught on a particular subject.

If we're going to play this role, if the Board says we're not going to play this role, then let's abandon it. If we're going to embrace it, let's be at the cutting edge of education and let's own it.

DR. SELBY: Your points are all very well taken, and I'm sorry the folks that know the most about this statement of work are not here at the moment, but we will get back to you very quickly,

and I think strongly consider your suggestion that if the scope of work doesn't include materials for course work, we can expand it.

DR. KRUMHOLZ: To give people warning this stuff is coming up.

DR. SELBY: And the warning. If I were a teacher, I probably wouldn't like enroll students thinking that PCORI is going to get the slides to me in time for the first lecture. Your point is very well taken, all of them.

CHAIRMAN NORQUIST: Barbara?

DR. McNEIL: Robin, the content of this course, is that something that was designed by your committee and then Hopkins has bid on the execution of the content, or are they designing the content, and if so, with whom?

DR. NEWHOUSE: This is one of those issues where the Methodology Committee said we need CME, so we did not develop the content nor the CFA.

DR. McNEIL: I misunderstood. I thought we were talking about two things. One is CME course for clinicians and one an academic course.

I was talking about the latter.

DR. NEWHOUSE: This was one of the activities where we charged the PCORI staff to complete the activity but we actually weren't involved in the development of the CFA or working with selecting the contract.

DR. McNEIL: What I'm trying to understand is is Hopkins developing the content or did the staff with the advice of others say this is really what we want, you go develop the course and materials for Harlan?

DR. NEWHOUSE: Yes.

CHAIRMAN NORQUIST: Jean?

MS. SLUTSKY: I'm more than happy to share the CE/CME. That is what I was involved in.

CHAIRMAN NORQUIST: She's talking about the graduate course. That, we don't know. We don't have the people in the room that know; is that correct?

DR. WALKER: This is Kara Walker. It was written around getting an RFP for the 11 methodology standards. It will be curricula

developed for different audiences. We are planning to collaborate with AHRQ so we are not duplicating efforts they are working on. Right now we are negotiating that contract so we can't share a lot of the details, but we certainly will have it negotiated in about two to three weeks and we can talk more about the specifics of what is being proposed.

Each contractor actually submitted a different level and expense of how they plan to distribute the curricula, what they plan to share more broadly, how they are going to do distance learning, et cetera.

DR. McNEIL: The Methodology Committee and the staff know infinitely more about what is to be included in the contract of this activity. Is the cart before the horse here?

CHAIRMAN NORQUIST: Robin and I actually had the same conversation earlier. I was wondering how come Methodology didn't just have the content and then let someone execute kind of the teaching part. I don't know the answer.

DR. WALKER: Part of the RFP and development is actually to go through a process where we vet the content and the curricula that's proposed, so we had input from Methodology Committee members and some other teachers of the methodology standards along the way. That is why there is a time lag, because we do want to make sure everyone agrees with what is proposed in the curriculum.

CHAIRMAN NORQUIST: The Methodology Committee will be involved with that. My impression was also, and I might be wrong, that they were supposed to be using the methodology report as a template for helping them, they wouldn't be restricted to that, but that would provide a lot of the background material. Isn't that right?

DR. WALKER: Exactly. We did give some general parameters around what the curriculum should include. Obviously, the report is one, but additional work will need to go into making sure it doesn't overlap with existing resources and

available information. There will be not only slides that people can use but also resources and reading materials, sort of additional supplementary materials that people can refer to.

CHAIRMAN NORQUIST: Leah?

MS. HOLE-MARSHALL: Thank you for the presentation. As we discussed a little bit, and it wasn't in your materials but it was in some of our other background materials about adherence to methodology standards. I didn't see it as a separate work group or activity here, so can you speak to that?

DR. NEWHOUSE: Yes. So, once the standards were formed, the adherence was taken over by -- the evaluation of adherence was taken over by the PCORI staff. They have been reporting back to us on the adherence. Actually, adherence is pretty good. There is a lot to be excited about and to celebrate.

There are a couple of areas where the adherences are a little bit lower, and they have been focusing on that area. I think in terms of

the continuous evaluation, they deserve a lot of credit for being able to interpret the adherence and the adoption.

MS. HOLE-MARSHALL: I was also impressed by that, and just wanted to make sure there was either a group or the Methodology Committee as a whole was reviewing that to see if there needed to be some adjustment in the methods or that the language makes that clear for researchers or if this is something we need to have a little bit more focus, but it looks like it is coming along very well.

DR. NEWHOUSE: Yes. There was a lot of discussion around the areas that were lower, heterogeneity of treatment effect was one of them. In fact, we just reviewed that standard for heterogeneity of treatment effect. The staff are coming back with the evaluation, there is a full discussion so that we can brainstorm ways to improve the adoption of that standard.

MS. HOLE-MARSHALL: There are some that might have ranges. I think it would be important

to get the Methodology Committee to make sure they are commenting on that, like definition of an appropriate question, things like that.

CHAIRMAN NORQUIST: Gail?

MS. HUNT: I just wanted to ask if there are any patients --

DR. NEWHOUSE: Let me check on that. We are going to have an update. We have a Methodology Committee meeting on Wednesday. We will get an update and I can let you know. If they are not, they should be.

CHAIRMAN NORQUIST: I think the point is they better be on there.

I will come back over here to Sharon.

DR. LEVINE: Just to maybe restate the question that I'm not sure I understood the answer to. What skill set is Hopkins bringing to this? If the content is PCORI content, what is the skill set that enabled them to win the contract? The second part of that is will the material, the curriculum, be copyrighted and if so, who owns the copyright?

CHAIRMAN NORQUIST: Wow.

DR. NEWHOUSE: Kara is coming back on that question.

CHAIRMAN NORQUIST: Where are we in the process of the contract right now?

DR. WALKER: The contract is under negotiation. We expect to finalize it in the next two to three weeks. We would be happy to share more details once we finalize the contract. The teams were very impressive. I think one of the things the Methodology Committee asked for is that this would be developed in a quick time frame, and it wasn't something we could do internally, and certainly wasn't something the Methodology Committee itself -- we were really looking for a group of qualified investigators, methodologists.

The copyright, they know it would be something that is shared openly, posted on our website that people can download.

DR. LEVINE: It will not be copyrighted?

CHAIRMAN NORQUIST: You can't have a copyright, right. Mary?

MS. HENNESSEY: This is Mary Hennessey, PCORI's General Counsel. From what Kara is telling me, this particular contract is not yet finalized, but just generally speaking about issues about intellectual property, generally speaking, there are a number of approaches to ensure that the public can have access to it.

That can include PCORI owning materials and letting others have a broad license to it, but it is also a very acceptable model to have someone else own, and we have a non-exclusive unencumbered license to use it and disseminate it.

There are a variety of legal models that you can pursue to reach your goals, and in this particular contract, it sounds as if we are not quite final but we are very attentive to ensuring the goals about PCORI's ability to disseminate materials.

CHAIRMAN NORQUIST: Okay; thanks.

DR. LEVINE: To make sure I understand the answer, the process then relies on the contractor to ensure the integrity of the translation of the

methodology standards and curriculum. Is that part of the iterative process you were describing, Robin?

DR. NEWHOUSE: Kara, I think you will have to answer how your intent was to interact with the Methodology Committee, but I think what I was explaining was our experience with the CME activities. We have not yet started to interact in any way on the academic curriculum. I'm going to defer to Kara.

DR. WALKER: That's right. The contract is still under negotiation and the work has not begun. We do expect input, as well as from the community that is working on PCOR training materials.

CHAIRMAN NORQUIST: Bob?

DR. ZWOLAK: This changes the subject --

CHAIRMAN NORQUIST: Okay, wait a minute. Allen, do you want to be on this subject? Steve?

DR. GOODMAN: Part of the question is the qualifications of the team. I don't know the full team that applied, but my impression was it was led

by Jodi Segal and they run a fellowship or CER training program, one of the few. They have been teaching this for a while, if I'm not mistaken. They have a lot of independent expertise on this team. Isn't that right, Kara?

CHAIRMAN NORQUIST: I think she has to be careful about what she says at this point since they are negotiating the contract.

DR. GOODMAN: Okay. I just wanted to emphasize that I think they are independent scientists and investigators with a long track record in both teaching and doing comparative effectiveness research.

CHAIRMAN NORQUIST: All right. Thanks. Bob, you're going to change the subject?

DR. ZWOLAK: I'm going to change the subject. My question is on usual care. We have had discussions in the SOC about usual care. To some extent, while I'm delighted that you are focusing on it, it seems like a lot of studies are already underway or funded and ready to start.

You are going to redefine "usual care,"

and my assumption is that going forward with studies that we fund, they will use your definition of "usual care." Is there any way, either retroactively, that studies that perhaps have been funded but haven't been started, once you make your definition, or even studies potentially underway now, are going to be able to use your definition of "usual care," or will we have studies underway that contradict our own PCORI definition of "usual care?"

DR. NEWHOUSE: Hal, do you want to answer that one?

DR. SOX: I guess in a way it depends on what our policy is and whether it is one that could be implemented retroactively. The SOC discussed a draft policy which basically said you need to define the content and you need to measure it, something I think I heard earlier today. They seemed to like that proposal, and we have actually put it into a recent funding announcement.

The Methods Committee is going to have a work group to try to get some more depth to that,

perhaps change it, if it doesn't make sense on more careful examination, and hopefully write an article that would be very influential.

That's the plan going forward. We obviously can't require people to collect data about the details. Basically, what we are saying is we want to find out what care each patient got so then we will know what usual care is, and that seems unrealistic to apply retroactively.

CHAIRMAN NORQUIST: Other questions for Robin?

[No response.]

CHAIRMAN NORQUIST: Thank you and the Methodology Committee, and welcome to the new member. Thank you all very much. Jean, you're up. Bob, did you want to make a comment?

DR. JESSE: The fellow who once said there's no such thing as usual care, it's random care.

CHAIRMAN NORQUIST: Jean is going to talk about the communications dissemination research program.

MS. SLUTSKY: Thank you. I know we're running short on time. I'll try to be quick. I'm just going to talk a little bit about the program as it is now and introduce the team, as well as give you an overview of the portfolio and our 2015 goals, and then we will have some time hopefully for some questions and answers, but I know we are running short.

I came here about February 17 or 18, and there was no CDR team. The first thing was to hire up, and what you see is the team. Rachel Melo, who is my executive assistant, is not on there, but she also serves as staff assistant for the team.

We have people on the team that have expertise on shared decision making, decision aids, community participatory research, and theoretical concepts of communication and dissemination research.

An overview of the portfolio, before we talk about that, this background really is the premise for the CDR portfolio based on the fact that patients, caregivers and clinicians need to be

equipped with the best available evidence when making informed decisions, and knowledge about how to optimally communicate and facilitate effective uses of evidence, information and tools that is lacking in any area.

We need strategies to make existing patient-centered outcomes research and information available to patients and providers and to make the dissemination and implementation of this knowledge useable in various contexts.

The CDR portfolio is one of the five national priorities that were established by the Board of Governors in 2012. This program seeks to fund comparative effectiveness research, so we want to fund research that is comparative in nature, that involves the direct comparison of effective health communications dissemination interventions or strategies that engage patients, caregivers, and providers, in the context of real world clinical care settings and situations, the natural course of where people get care and how they would make decisions about their care, and to enable patients

and caregivers to make the best possible decisions when choosing among available options for their care and treatment.

The CDR funding is around three key areas, communication strategies, and what we mean by that is to promote the use of health and health care CDR evidence by patients, clinicians, and others.

Different dissemination strategies, and we mean by that to promote the use of health and health care CDR evidence by patients and clinicians.

A third one which is a little bit different than you might have seen in other CDR portfolio's, which it explain uncertainty in health and health care CDR evidence to patients, clinicians, and others. I will speak about each of these in just a minute.

For example, I'm just using three funded projects, just to explore different ways that we have funded applications based on these three goals. This is a communication strategy example. It is titled "Amplifying the Patient's Voice,

Patient-Centered Versus Measurement Based Approaches to Mental Health." This study compares two ways for patients and prescribers to engage in shared decision-making around medication and treatment appointments in community mental health centers.

It compares patient-centered care using a decision aid with a navigator, sort of like a navigator of care counselor, versus measurement-based care looking at symptomatology and how that impacts adherence to medication.

It examines differences in outcomes depending up on an individual's experience with medication treatment, the level of intervention use, and the severity of their mental illness.

This study is in 3,000 Medicaid enrolled with mental illness who receive medication at 1 of 14 community health centers across the State of Pennsylvania.

A dissemination strategy example is comparing traditional and participatory dissemination of a shared decision making

intervention. This actually evaluates three different alternative approaches to dissemination of an evidence based shared decision making toolkit, and it aims to determine what dissemination strategy most effectively increases practice level adoption of shared decision making, improving patient outcomes, and increasing patient involvement in care decisions.

Again, a comparative study looking at three alternative approaches.

It utilizes a partnership between a statewide Medicaid network and the North Carolina -- I'm sorry. I don't know that acronym is now, my fault.

As you can see, both of these studies look at under served and under studied populations.

This is a study that is Explain Uncertainty, and this is a really interesting study that describes the comparative effectiveness of colorectal cancer screening tests, looking at the impacts of quantitative information.

It is evaluating impact, doing

quantitative information, and a decision aid on screening behavior and perception of risk in patients eligible for colorectal cancer screening. Looking at different mechanisms for explaining risk using concepts, sometimes it is done graphically, sometimes in different types of pictures.

It allows people to see what their individual risk is using a variety of different formats. It also uses the public deliberation exercise to review the results of the clinical trial and makes recommendations that have decision aids to present quantitative information to patients.

A very unique aspect of this is the public deliberation and involvement with the public in looking at the results of the study and making recommendations.

I just wanted to give you sort of a view of what disease and conditions are covered by the portfolio. This is very similar to other portfolio's, the other national priority areas at PCORI.

Very heavy on mental health, behavioral health, disorders, cardiovascular disease, and cancer. The large other category includes diabetes, some radiologic interventions, and rare diseases.

We also have a pretty broad area on priority populations, although we don't have as many veterans involved in our studies as I would like to see, but we have quite a few racial and ethnic minorities and low income populations that are included in our study.

I just want to talk a little bit about decision aids because Hal Sox presented to you I guess a little over a year ago, almost two years now, about the fact that across PCORI, we were funding a great deal of decision aids development, so not comparing whether or not decision aids worked better than other strategies for communicating comparative effectiveness research but actually the development and validation of tools.

Right now, our portfolio is made up of 34

percent decision aids/tools and about 66 percent are non-decision aids or tools. Part of this is a reflection of as I've staffed up the portfolio, we have continually refined what we're looking for and been very clear of what we're looking for.

We have had a moratorium on the development, testing, and validation of individual decision tools. We have said to the community we are not doing tool development, and what we mean by that is a very large part of the applications are budgeted to develop and validate, and what we are really interested in is looking at how these tools and other mechanisms compare for communicating and disseminating complex information.

That has been reflected in a lower percentage of actual development of tools in the portfolio.

We also developed an organization wide decision aids work group. This was based on the fact that we have a large number of these tools in the portfolio. The idea was to conduct an extensive and exhaustive search to identify,

categorize, and describe what we actually have in the portfolio across PCORI.

The findings from this study will really help us further refine the strategic portfolio development as well as future funding announcements more targeted funding announcements, because of deficits in the portfolio or in the organization.

We expect those results to be made public in the coming months.

Some goals that we have had for 2015, and I am almost done, for those of you are thinking it is getting late, we are launching the inaugural on the CDR Advisory Panel. Their first meeting is in a couple of weeks. We are continuing to refine the CDR portfolio PCORI funding announcement.

We are also considering, now that we have the staff and expertise, larger, more targeted announcements, like hosting or managing a CDR relevant pragmatic clinical studies, and many of our working groups, both for Hepatitis C and MS, the whole concept of shared decision making in these areas was rated fairly highly by the

participants, so scoring this with the other portfolio's will become a priority for us.

Also, exploring opportunities for priority topics in targeted funding, especially around dissemination of robust CDR findings where we have identified gaps in the portfolio now that we have the people power to actually look at what we have.

Also, because we have significant expertise in our staff in the CDR portfolio, we will be contributing to the dissemination and implementation framework.

This is sort of where I take one hat off and put another one on, to help further define the PCORI conceptual dissemination framework that I will be discussing with EDIC tomorrow to develop limited competition funding announcements among PCORI awardees to allow them to disseminate PCORI research findings on their studies, and to create the infrastructure to translate and disseminate robust CDR findings for different audiences, in coordination with AHRQ, which we continue to meet with on a regular basis.

CHAIRMAN NORQUIST: Thank you, Jean, for that review. Questions? Comments? Barbara?

DR. McNEIL: You were talking about public deliberations in one of your earlier slides. What exactly does that mean?

MS. SLUTSKY: It's part of the study on quantitative presentation of information on colorectal cancer screening, and it is part of communicating uncertainty of results to the patients.

They aimed in the latter part of the study to use a public deliberation exercise to review the results of the study and have them make recommendations for how the decision aids represent the quantitative information.

DR. McNEIL: I thought there was so much counterintuitive information about the impact of the various ways to present the data and the impact of various ways to present the data have been documented by a diversity of people for decades -- I'm not sure -- because of all the various risks and biases that comes into play.

MS. SLUTSKY: It's interesting. AHRQ under their ARRA funding funded a fairly large randomized control trial using public deliberation. There are some pretty good findings that show that if it is done well, public deliberation can actually through an iterative process be very informative about the best way for people to actually take in information based on being able to ask questions of experts before they actually make their decision. It's run much like a jury would consider evidence in a trial.

It's an interesting concept used a lot in education and environmental policy, where do you shut down a school, where do you put toxic dumps.

CHAIRMAN NORQUIST: That is an interesting analogy.

MS. SLUTSKY: Especially today, right.

CHAIRMAN NORQUIST: Sharon? Harlan?

DR. WEISMAN: Jean, I think this is really important work. I was at a conference a couple of months ago and was surrounded by a group that was very upset about our efforts in dissemination

because by statute, we were specifically supposed to not touch or do anything with guidelines, treatment guidelines. I don't think we are.

MS. SLUTSKY: It's an ambiguous line.

DR. WEISMAN: It can be an ambiguous line, and I'm just wondering what your thoughts are on the distinction between what we are doing and what by the law we are told we cannot do.

MS. SLUTSKY: Actually, we have a page on the application process where it is sort of a check off box, that's an elegant way of saying it, but we ask applicants if they are developing cost analyses, if they are developing clinical practice guidelines, if they are developing a tool, they automatically get put out of the system.

I also would also add kudos to the CDR team. They go through the applications, particularly the budget, with a fine toothed comb, and we have actually identified some applications where they have a line item to develop a guideline that didn't come through in the write-up. We have excluded those applications as well.

The majority of decision aids do not make recommendations for care, and most of them present the options to you along with your personal preferences. Those are the types of activities that PCORI is funding.

CHAIRMAN NORQUIST: Other comments?

[No response.]

CHAIRMAN NORQUIST: Thank you, Jean, very much, doing a wonderful job, so is your crew, too, we should thank them also. I just wanted to say you started out saying you have been here since February 17, a year ago, so over a year you have been here. Thank you.

MS. SLUTSKY: I'm sorry; right.

CHAIRMAN NORQUIST: I hope it hasn't felt like --

MS. SLUTSKY: Math has always been my problem.

CHAIRMAN NORQUIST: All right. Thanks. Last but not least, Christine is going to present on the topic prioritization process. There should be some discussion about this, I suspect.

DR. GOERTZ: Thank you. Today we are going to talk a little bit about identifying topics for our prioritization process, both for our targeted PFAs and for pragmatic trials.

We are pretty familiar already with what we have been doing thus far. You will remember we spent a lot of time talking about this at our retreat a little over a year ago, I think just slightly before Jean joined us, and then we ended up voting on it, on the prioritization process, in July.

Just really briefly what that process includes is an initial review of submitted topics by the staff -- the review is by staff of topics that have been submitted by various stakeholders, and then there is a preparation of topic briefs on a subset of those topics, usually that has historically been done by outside research teams.

That is followed by a review and prioritization by our multi-stakeholder advisory panels, and then the SOC has looked at that list of topics and approved them to move forward to the

next stage, which would be multi-stakeholder workshops on selected topics.

Normally, by the time we get to this point, we are using the term "topic" because we haven't necessarily honed in on a specific research question at this point, it's still a little bit more broad, and we have used the multi-stakeholder workshops to try to take a more broad topic area and hone it down into a little bit more targeted research question, which has then gone forward in terms of a targeted PFA or else has gone on our pragmatic studies high priority list.

Just to give you an idea of how that works, gives a little bit of a graphic of that. As you know, there has been some questions about how this actually works, and why is it we are not getting more targeted PFAs out the door.

The SOC has debated and the Board has debated at various times about to what extent should be pursuing investigator initiated research versus being even more targeted. In fact, we had a little bit of that discussion today already.

What I'm going to do now is talk a little bit just to remind you about what some of the steps are within each of these categories, and then talk about some updates that we have made to this process.

Basically, in order to determine topic eligibility, the staff used some Tier 1 and Tier 2 review criteria, which you have already seen, but I'll review really briefly in just a minute.

What we are proposing now is that the Science Oversight Committee would actually get involved at a point that we currently don't, which is between this relatively early determination of topic eligibility and before topics actually go for topic briefs.

The topic briefs are actually quite a lot of work, and there are some costs associated with those, and there have been instances when something has come to us after the topic review stage and we have been unclear in some cases and in some cases fairly certain that wasn't really an area that was of high programmatic interest to us for one reason

or another, or have been concerned that maybe the questions that were developed through the topic briefs weren't specific enough or quite on target.

We would add the touch points before the topic briefs are developed. Over almost a year now, the SOC has reviewed the topic briefs after they are developed. We have continued to do that. After that, they would continue to go to the advisory panels that use Tier 3 criteria to review and prioritize the research questions, and I'll cover what those Tier 3 criteria are in just a minute. Then the SOC would be more proactive in selecting topics for further development.

One of the parts of this process that hasn't worked probably as well as we would have liked is really probably in this particular phase of the game. At this point, prior to now, even though we did have an opportunity to review the topic briefs, the staff has generally put a lot of work into what topics they are selecting for further development before they are presented to us.

Sometimes I think that has led to frustration on both of our parts, that there has been a lot of work put in and then we are unclear if that was a topic we were really interested in or maybe it was a topic we were interested in but that might not be exactly the research questions that we had thought was most important.

Also, there have been questions about how is it that a topic or research question actually arrived at this stage of the game, and I would say we have had right around 1,000 topics that have been submitted thus far, and I don't think we -- I know we have not been completely transparent about exactly what has happened to those topics as they have gone through our process.

A lot of what we are talking about here is really the process in order to be more transparent and to make sure we are not really getting a backlog or getting frustrated at this particular point.

Then the process would include a review of what we are calling our Tier 4 criteria to assess

the more specific research questions, and then we would work with staff to decide whether those research questions should best move forward as part of our pragmatic clinical trials, targeted PFAs, or as a targeted PFA.

What is happening is there are several lists that get produced, actually seven lists in each of the stages. Tier 1 and Tier 2 review criteria produce list one, and again, I'll about what their criteria include in just a minute.

After the topics are selected for topic briefs, that would be list two. After the topic briefs are reviewed, it would produce list three. After the advisory panels had an opportunity to look and prioritize the research questions, that would produce list four. The SOC would select topics for further development and the work groups would refine the questions, which would produce list five, which would then be more or less divided into lists six and seven.

It's all really clear now, isn't it?

CHAIRMAN NORQUIST: You didn't say, just

curious, the time line, how long it takes. Seems like this could take an eternity sometimes if you go through all this stuff.

DR. GOERTZ: I think this will actually streamline our processes and enable us to move forward more quickly. We have a little bit of a backlog right now. I believe list four is our longest list right now. Is that correct, Kara?

CHAIRMAN NORQUIST: I'm sorry, you mean takes the longest?

DR. GOERTZ: No, right now, that is where more of our backlog is, because remember I said that tends to be where we have had a little bit of things have gotten stopped up. The plan is we would be moving fairly rapidly through these lists as they get developed in the future.

Joe, did you want to say something?

DR. SELBY: Only that list four is the list that came back from the advisory panels. The difference, as Christine said, is we would bring these things back from the advisory panels almost before the SOC had heard about them, and that is

the back up. Now, we are involving the SOC very early on before we have done hardly any work on them. I think that is going to make a difference.

By the way, Christine, you just have to trust these slides because pretty soon they are going to pop back -- if you push the bottom and go through Tier 1, 2, 3, then you will get the next part of the flow sheet.

DR. GOERTZ: I see. Sorry, I'm just not that sophisticated. It never occurred to me to actually do the presentation. All right.

I guess we're going to be talking about our Tier 1 and Tier 2 criteria now. Really asking some very basic questions about whether or not this is really a comparative effectiveness research question. By that we mean two or more options, one of which can be usual care, which we talked about a little earlier, being compared, and does the study fit at least one of our national priorities for research. Those are the things that get you through this first priority level.

We determined a question is ineligible if

it's a common or descriptive question or a question of disease causation or biological mechanism, which comes to us sometimes, or if the study involves cost comparison or cost effectiveness analysis.

We are also asking if the question is duplicative with another question that has already in our topic database and is the question patient-centered.

CHAIRMAN NORQUIST: Since you're going to go through all of these and at the end we are going to have comments, you might want to just have some questions as we go.

DR. GOERTZ: That's a good idea.

CHAIRMAN NORQUIST: One thing I was just going to ask where you say does the study involve -- it's not that it involves, because it could involve and you could take that out and still have --

DR. GOERTZ: I think that's probably just not as clear wording as it could be.

CHAIRMAN NORQUIST: Right. I think what needs to be clear -- I want to be clear with the

Board that this is your first filter. If you don't make it through this filter, you're not going as a topic below this.

DR. GOERTZ: Right.

CHAIRMAN NORQUIST: We have to be clear that we are all right with these being our first part of the filter, that you have to get through this. Does anybody have a problem basically with these particular criteria being our first filter? Sharon?

DR. LEVINE: I'm not sure of that last exchange between you and Christine.

CHAIRMAN NORQUIST: I was saying on that bullet it says "Does the study involve," I think what it should say is the central question, does it focus on, because you could have a study in which you have a question about let's say comparison of like, for example, an antipsychotic, and you had a component that was cost effectiveness, you could pull the cost effectiveness out and still have the comparison of the antipsychotic.

Do you see what I mean? That topic might

still fall through for the next level of the filters.

DR. GOERTZ: Now that I'm actually thinking it through, if the question itself at this point is primarily focused on cost effectiveness, we probably would weed it out at this point.

CHAIRMAN NORQUIST: That's what I'm saying. If you had it as a component but it was not the central focus.

DR. GOERTZ: This would be too early for that component thing.

CHAIRMAN NORQUIST: Sharon?

DR. GOERTZ: Did that help?

DR. LEVINE: No.

DR. GOERTZ: I'll talk to you about it later.

CHAIRMAN NORQUIST: Harlan Weisman, and then Allen. We are focusing on this Tier 1 criteria.

DR. WEISMAN: This is an old question/ comment, and I've heard Barbara a few times talk about it doesn't compare A versus B, it shouldn't

be PCORI. I'm just wondering, and particularly in PCORnet, there are certain observational questions that might be important to learn about, you know, from a longitudinal basis or even a cross sectional basis, which would be informative and important to know to patients, clinicians, and other stakeholders, which may not involve A versus B.

DR. GOERTZ: Harlan, this really is focused on those questions where we would write a targeted funding announcement or put it in our pragmatic trials, not necessarily what we might be doing under PCORnet.

DR. WEISMAN: Okay. Was I supposed to know that? Was that obvious?

CHAIRMAN NORQUIST: You are being told now, I guess. PCORnet may have topics that don't necessarily fall through these filters. Is that what you are saying?

DR. WEISMAN: The seed money.

DR. GOERTZ: The endpoint for this is either a targeted PFA or a pragmatic clinical trial, sort of our --

DR. SELBY: These are really our big ticket projects.

CHAIRMAN NORQUIST: Big ticket in the sense of the way they are done, not through PCORnet.

DR. SELBY: And investments.

DR. WEISMAN: By the way, I agree that most of what we do or the vast majority would be of the sort you're talking about, but it wasn't clear to me you were only talking about those big things.

CHAIRMAN NORQUIST: So, this is very helpful for us to understand what is going on, right, so we are very clear on how the filters work.

DR. GOERTZ: Right. In answer to your question, I find myself just a little bit less clear.

CHAIRMAN NORQUIST: All right. Any other questions about this or comments about these criteria? Allen?

DR. DOUMA: Thanks. It's a process question, and that is if something doesn't make it

from list A to list B, let's say for example from list 3 to list 4, is it always out of the running? Does it get throw in the garbage, and we keep moving on, and that is true forever for that topic?

DR. GOERTZ: We don't actually throw it in the garbage, we just choose not to move that question forward as a priority. It doesn't mean some other version of that topic might not be a priority, or it doesn't mean things might change and it's possible the environment might change in some way which would change our prioritization of that.

They don't get thrown away, they just stay on a list and don't move forward. They always have a chance. You remember the fairly elaborate graphics that we had last year when we talked about this, they can always be more or less cycled, but something would have to change in order for that to happen.

CHAIRMAN NORQUIST: Ellen?

DR. SIGAL: I guess maybe a clarification, maybe I'm waking up sleeping giants. I thought we

were not prohibited from dealing with costs. I thought the legislation made it clear, although it may not be our high priority, we were not expressly prohibited from dealing with costs. It's a question.

DR. SELBY: I think we have operationalized the language in the legislation based on our own good sense and on comments we have gotten from any number of stakeholders, so we say very clearly and have from almost day one, we will not support studies that focus on cost comparisons between two treatments or cost effectiveness analyses.

Even though you can quibble about what the language said, we have already operationalized it that way, and the only exception is that we have every interest in studying out of pocket costs, both how they differ -- to patients -- and how they drive behaviors, adherence, and outcomes.

Costs and others, we don't. We do, however, measure utilization, differences in utilization.

CHAIRMAN NORQUIST: Rick?

DR. KRONICK: On the previous question about if a topic doesn't make it from list A to list B, what does that mean. In part what it means depends on what difference it makes to be on the list at all. It seems clear the topic has to be on the list in order to become eligible for a targeted PFA, but they are probably not going to do a targeted PFA on something that hasn't gone through this whole rigmarole, advisory groups, topic briefs, et cetera.

For either the broad funding announcements or particularly for the pragmatic clinical trials, it's not clear it means all that much to be on the list, at least it's a question of how much preference to give to topics that are on the list versus not.

We talked about that a little bit at the very beginning, and probably something we should come back to.

CHAIRMAN NORQUIST: Yes, we should be clear about that. I think you're right about the

broad announcements. I'm not so sure about the pragmatic.

DR. SELBY: We have refined the RFA, the PFA, a bit to be somewhat clearer that if you are on a high priority list, all other things being equal, you will fare better. That is the way to say it. It's true that topics we never thought of can knock our socks off and get funded through the PCS.

DR. KRONICK: Mike Lauer has kind of challenged that. We had some discussion within the SOC, although I think having come to -- the question hasn't really been called yet, haven't been asked to opine, but from his many years of experience, he would argue NIH is not so good, I think, that it's very difficult to figure out what the topics are that are going to result in research that really has a big effect, and we should leave it much more open to the field and trust in the peer review process, having first stated very clearly the principles of what kind of work we want.

DR. GOERTZ: I think we have had that discussion. I also think in the latest version of our research strategy that I think we need to continue to revisit annually based on budget availability and other issues such as this, it does say right now that we want to focus, and we would be placing higher program priority on those applications that are on our lists.

That doesn't mean that we can't revisit that, but right now, that is what our research strategy says.

CHAIRMAN NORQUIST: Barbara and then Bob Zwolak.

DR. McNEIL: I was part of the meeting that Rick just talked about, and Mike was there. It strikes me that we really should be talking about this more. At the moment, I think we have a fine set of lists here. I do have a lot of sympathy with what Mike Lauer said and what Rick just annunciated very clearly.

I do wonder if we are not over determining the system, this very thoughtful but very deep

list. I think we should talk about it in this group as a whole, and I wonder if fact -- I personally would like to see Mike's data.

You really have to ask whether with all the lists in the world and with as much input in the world, we are going to get more information or better questions from the oncologists at Sloan Kettering who really has the immediate on the ground question about A versus B for melanoma. It just might be that we never thought of it at the time we started list 1.

I'm not faulting this at all. I'm just saying I think it's time after three years that we really rethink -- not that we would change it -- at least go through the process of deciding whether over determining things is the right way to go.

DR. GOERTZ: One of the SOC -- as you know from the last time we met, we decided to pull together three work groups, and one of the work groups was focused on our research strategy and what changes we might want to make based on changes in funding, et cetera. I think that would be a

good conversation for us.

We did have this conversation in quite a lot of detail three or four years ago when we first started talking about what it is that we wanted to fund, and in fact, when we did the broad announcements, at that point our strategy was that we would not be specific and we really wouldn't put things on the list, and over time, we have made the decisions to incrementally be more targeted in a number of ways.

That doesn't mean we can't revisit that, but we have been moving from a position of really being most interested in broad investigator initiated research to being increasingly more targeted over the last three years.

CHAIRMAN NORQUIST: Bob?

DR. ZWOLAK: Thanks. I'm getting confused about this now. Even being on the SOC, I'd like some clarity. It's been my understanding that we have and will always have a category called the "broad funding announcements," for which these lists are completely irrelevant.

There could even be a topic on list 1 that never got past list 1, and if someone sent in a fabulous application under the broad announcement, that could be funded; is that right?

DR. GOERTZ: Correct.

DR. ZWOLAK: Really, the issue then is I think the relevant proportion of funding that we allocate to these two pathways.

DR. GOERTZ: Right.

CHAIRMAN NORQUIST: Ellen?

DR. SIGAL: To kind of confirm what Barbara was saying, what we have been talking about, broad versus narrow, I find that if you go to the gaps, where there truly are these questions, and if you go broadly, you just do not get the information. If you go narrow to the people who are really in the field, in the trenches, who know these questions, you get much better and much more actionable projects.

I can tell you just recently spending four months on this, going broad gave us nothing, absolutely nothing, even when we had a very

specific -- even when we went in disease specific groups to work on something, and came up with nothing. Recently, when I have gone to the narrower group studying a particular disease, generating lots of projects and most of the ideas coming through. I think that is something we have to think about, particularly where there are gaps.

CHAIRMAN NORQUIST: Joe?

DR. SELBY: If the broad's were yielding a large number of head to head comparative studies and all we had to do was pick from them, then you would be absolutely right, but as Christine said and as Ellen said, the broad's tend to solicit research about patient-centered care almost exclusively.

This is a process. First of all, it's a process to get to the targeted funding announcements, the actual specific ones, but remember, they start by coming from stakeholders. They start by coming from the bright oncologists at Sloane Kettering or from a patient organization or payer.

The number one purpose of this whole prioritization process and for convening the advisory panel is to get these really targeted topics that the stakeholders we were set up to serve have brought to our attention.

What is happening is that enough questions like that come through that we couldn't possibly write targeted announcements on every one, and that is a part of the role that the PCS, the pragmatic clinical studies, play, topics that have done quite well in our advisory panel that have been brought to us by important stakeholder groups that didn't get all the way to a targeted announcement, can be singled out as of particular interest to us, and substantial monies set aside.

I think we need to keep both of those purposes of this in mind.

CHAIRMAN NORQUIST: Okay. Rick?

DR. KRONICK: I think everyone agrees there needs to be some process for getting to targeted PFAs, and this seems like a good basis of that process, although I do note that at least for

some of the most recent targeted PFAs, it's only partially resulting from this process, or at least it's not clear to me how we got from 22 potential topics to the particular targeted PFAs that we have recently received.

DR. GOERTZ: That is one of the reasons why we are going through this process. We really are trying to better operationalize a process that we said we were going to put in place almost a year ago, and to make it much more transparent.

I don't think there is anybody in this room who would be able to say oh, I understand exactly how everything has gone through the process.

DR. KRONICK: In that last branching, as we branch from list 6 to 7, targeted PFAs versus a topic that could be part of a PCS, that is still a very tough thing to figure out how to do in a transparent and open way.

Bob, to your comments earlier, I think it's not simply a question whether it's broad or not, because there is also this question of how the

pragmatic clinical studies are going to be treated, and they could be treated as a broad announcement, that we are interested in pragmatic clinical studies that have the following characteristics, or they could be treated as a more targeted announcement to say all the characteristics, and we want one of these 22 topics. It's that kind of open question.

DR. GOERTZ: I think one thing to keep in mind with the pragmatic trials is investigators still have the opportunity to come to us with an idea that's not on any of the lists and still do well. In fact, I don't know how many of the ten that we have funded fall into that category, but at least three of them.

The fact that we are prioritizing this and coming up a priori with our priorities, we are still leaving it open saying there may be things, that we recognize that we don't necessarily know all of the great questions out there. It is still open for that. It's just that at the same time we are trying to put some structure to it to better

signal to people what it is that we are interested in.

CHAIRMAN NORQUIST: You make it through this filter, what happens next?

DR. GOERTZ: You go on to Tier 2 criteria. The first screen was really pretty basic, and this screen is a little bit more involved. It's looking at what is the impact of the condition and the health of individuals and populations, looking at whether or not there is important evidence gaps.

You could have a really great question but maybe that question has already been answered or there is a study in process that would be able to answer it before we could get anything up and going.

Is it probable that what we funded would help to close that gap. How likely is implementation, do one or more major stakeholder groups, for instance, endorse the question.

Some of these questions are based on initial consultations with patient clinician or other stakeholder, researcher funding agencies.

At Tier 1, staff are more or less by themselves making the decision about whether they meet some really basic criteria. At this point, we are starting to bring in other people to talk about some of these issues and make some of these determinations.

CHAIRMAN NORQUIST: Questions? One thing I would ask, when you are looking at process, are you looking internationally at what others may be doing or just an U.S. survey to see who is funding or whatever?

DR. GOERTZ: I'm assuming that we are looking more broadly than just the United States, but I would have to ask Kara or Brian to confirm that.

CHAIRMAN NORQUIST: I just came back, and the Australians were there, and they were talking about some of this. It is something to think about, right. We should not be duplicating stuff, particularly for some of the questions that could easily be answered in other questions. I'm thinking about current funding, where a question is

starting to be answered by somebody.

DR. GOERTZ: Any other comments on this?

CHAIRMAN NORQUIST: Those criteria get you into the second bubble basically?

DR. GOERTZ: That gets you to list 3 -- I'm sorry, list 2. I'm sorry; yes. Actually, Tier 1 and Tier 2 criteria bring you to list 1, and then what happens is the topics are selected for topic three.

CHAIRMAN NORQUIST: That's what you're talking about now, the SOC will insert itself at this point to decide what of these topics that came out of the Tier 1 and 2 go into a topic brief?

DR. GOERTZ: Exactly. What topics are we going to put more time and energy into developing, again, before a decision is made. That will produce list 2. After the topic brief --

CHAIRMAN NORQUIST: I'm sorry. I hate to keep harping on this, but to get to list 1, how long is that going to take? It seems like that shouldn't take very long, if we use those criteria, it should be a day or two, to get to list 1?

DR. GOERTZ: Right, that should be a fairly quick review, with the exception of those where we may need to consult with some other stakeholders or potential funders, it may take a little bit longer. It would move fairly rapidly through that.

CHAIRMAN NORQUIST: By this point, we should have culled the big list to get to list 1; right? What is feeding into this should be a lot fewer number of topics; right? Shouldn't we have in the last three years spent a lot of time with the IOM list and others trying to get into list 1?

DR. GOERTZ: That is correct. We started off with something just a little bit south of 1,000 topics that basically accumulated, but it is true that now the number of topics that are coming on to this new list are lower.

After the topic briefs are available, we review those topic briefs, and then based on those, some things we get more excited about after reading the topic briefs and some things we get less excited about. Those things that we are the most

excited about are put in the highest program priority will then move forward to produce list 3.

Once something goes on list 3, it would go to the advisory panel, and the advisory panel would use the Tier 3 criteria that we will be discussing in just a moment. Those things that were looked at unfavorably by the advisory panel would then produce list 4.

CHAIRMAN NORQUIST: I don't see it here. What criteria are you using to get on list 2 and list 3?

DR. GOERTZ: The criteria that you are using --

CHAIRMAN NORQUIST: What makes your decision to have somebody be a topic brief now? If you made it to list 1, now the SOC gets involved and decides what -- how do they make that decision? Based on kind of you like it? You know what I mean?

DR. GOERTZ: Right.

DR. SELBY: I don't think we have actually talked about this a lot, but I think it would be

really focusing on the Tier 2 criteria again by the SOC, who brings additional expertise, new eyes, new perspectives.

DR. GOERTZ: Right. It really is just looking again at primarily the Tier 2 criteria.

CHAIRMAN NORQUIST: I think you just need to be clear about that so they understand that the way you get in to get in a topic brief is that, and then when the topic brief comes back, are you still applying the same criteria to get on list 3 at this point?

DR. GOERTZ: That's correct.

CHAIRMAN NORQUIST: You have to apply some criteria.

DR. GOERTZ: You're essentially going back to -- you are looking at the topic brief and you are again applying criteria from Tier 2, but you have more information by which to apply it at that point.

CHAIRMAN NORQUIST: By the time you get there, the others may have already been picked up by somebody else for funding. How long does it

take to do a topic brief?

UNIDENTIFIED MEMBER: [Off microphone.]

CHAIRMAN NORQUIST: When you contract out, how long does that take?

UNIDENTIFIED MEMBER: [Off microphone.]

CHAIRMAN NORQUIST: Three to six months. Why are some getting contracted out and some are being done in internal?

DR. GOERTZ: I think we started moving more towards -- they were all getting contracted out, or I think most of them were getting contracted out, and now with this last batch, I think the staff basically did them; is that correct?

CHAIRMAN NORQUIST: Okay. That would obviously be a more efficient way of doing it, if we trust the staff to do it; right?

DR. GOERTZ: I think it really helped enhance the quality of the topic briefs, too. When you contract out for something like that, people can't necessarily read our minds. PCORI staff have a lot better sense of what it is we are looking for

and how we want the questions to be developed.

CHAIRMAN NORQUIST: I read some of the topic briefs. They were pretty bad, I mean, they were contracted out.

DR. GOERTZ: I was trying to say that very politely.

CHAIRMAN NORQUIST: I didn't mention who it was. Some were good, some were bad.

UNIDENTIFIED MEMBER: [Off microphone.]

CHAIRMAN NORQUIST: I think the criteria on that would be if you have the expertise in-house and you can do it, it's a much more efficient way to do it. If you really just don't have the expertise, then you contract it out, but you do it very quickly.

DR. GOERTZ: Obviously, there is a really big difference between two and a half weeks and six months.

UNIDENTIFIED MEMBER: [Off microphone.]

CHAIRMAN NORQUIST: You don't have to have a contract to do that, you can just call them up or talk to them; right?

DR. GOERTZ: I think one of the problems we were seeing with the topic briefs as they were being developed is we were basically hiring generalists to develop them that didn't necessarily have the expertise, and when staff are developing them, they are actually reaching out to content experts, and that's enhancing the quality.

CHAIRMAN NORQUIST: Okay. Thank you. Now we are up to getting to list 4. We are almost there.

DR. GOERTZ: Yes, now we're halfway there.

CHAIRMAN NORQUIST: I'm trying to count the time here, we are up to about a month so far.

DR. GOERTZ: Right. The advisory panel again, they come through the advisory panel and those things that are prioritized highly by the advisory panel would produce list 4. Then at that point, again, this is at the point where traditionally the SOC has been most involved in this process. We have continued to be involved in this process, but again, at this point we have already seen these topics several times, so we are

a lot more familiar with them. We are not just getting hit with something new, and we believe this will help expedite this process and allow us to move pretty quickly from list 4 to list 5.

Once we go through the advisory panel process and the SOC is saying yes to move forward, at that point it would go to work group that would further refine the questions if necessary.

I think we have relied pretty heavily -- at this point it is not uncommon for us to still have fairly general research questions, questions that may be too general to actually put into a targeted PFA, for instance, and the work groups can help us decide is there a really specific research question that we want to answer that would make a good targeted PFA, or should we consider something a little more general, which means it might be more likely to end up on the pragmatic.

CHAIRMAN NORQUIST: A work group is composed of?

DR. GOERTZ: Content experts.

CHAIRMAN NORQUIST: That we select?

DR. GOERTZ: Yes, or multi-disciplinary stakeholder panel, but that has some content knowledge.

CHAIRMAN NORQUIST: Are they helping to also refine the more specific question?

DR. GOERTZ: They are. They are extremely helpful in helping to refine the question.

CHAIRMAN NORQUIST: Okay. Sharon? You put her card up.

DR. LIPSTEIN: Now it has taken us a month to get to Tier 4.

DR. GOERTZ: It used to take us nine months to get to this point.

DR. LIPSTEIN: My question is in this process, once we get down to lists 4 and 5, does it ever happen where the Science Oversight Committee begins to substitute its judgment for the judgment of either the advisory panel or the research work groups, or is the Science Oversight Committee just governing the process to make sure we adhere to the process and we keep to our priorities and agenda?

The reason I'm concerned about that is

whenever you have a committee of a board or a board that has researchers and scientists on it, it can begin to substitute its judgment for the judgment of the scientists and researchers that you have put on those panels.

Now that I've joined the Science Oversight Committee, maybe I'll get to find out.

DR. GOERTZ: You will.

DR. LIPSTEIN: Can you give us insight into that?

DR. GOERTZ: Here's what I would say, it is mostly looking at the process and making sure that the process has been followed. I think that will be much more true as we have operationalized this new process.

I think there are times when we have brought as a committee our own thoughts and perspectives into this process as we were making decisions about what we might want to move forward with as far as a targeted funding announcement.

I would be curious to see what percentage of targeted proposals that have been presented to -

- I would say we have turned down, we have felt uncomfortable with relatively few pragmatic -- actually both targeted PFAs and pragmatic PFAs that have come to us at this point.

It's not unprecedented that we have said no, we don't feel comfortable moving forward at this point. Now, you have me curious about what those numbers have actually been, but what has happened is things have gotten sort of stopped up at this point, we have asked for a refinement of questions, asked staff to go back and further refine and hone things, and that has taken a little bit of time.

I think for the most part in the end, we have voted to move forward.

DR. SELBY: Although that's not necessarily a good thing. I think as the number of questions mount, you're going to have to say no more. I just want to point out, that is the Board's job in the legislation. It is the Board's job to decide what we are going to fund and what we are not, to create the research agenda portfolio.

I think it's much better to have the Board involved through the SOC early in the process than to get all the work done and staff chomping at the bit to write a PFA and the Board for some reason decides no at that point.

DR. LIPSTEIN: I agree, part of the way the Board has exercised its statutory obligations is to establish both advisory panels and a peer and merit review process. If the Board substitutes its judgment for the peer review, the merit review, the stakeholder engagement, or the advisory panel process, it's going to be hard to keep those folks engaged.

DR. SELBY: The second thing that Christine will get to hopefully by the end, if we have time, is precisely that, we are going to make this process much more transparent than it has been, so the community can be the judge of whether somehow we are slipping.

CHAIRMAN NORQUIST: That has been an issue because some people have asked how did you get to this topic, and the more we make it much more

transparent of how we got there, then it opens it up to some conversation.

DR. GOERTZ: Right. Steve, I agree with you about substituting Board judgment for all of these other processes, and to a certain extent, I would argue that the reason why we haven't done that a great deal is because thus far it's been pretty easy to prioritize in that we have had enough funding to move forward with all this.

For instance, right now the way it works with advisory panels, each of our programs have their own advisory panel. They each prioritize four or five different topics within those advisory panels a couple times a year.

We have been able to move forward with all that, but as our funding tightens up over the years, we're going to have to be prioritizing not just within programs but across programs. There just simply won't be enough -- there is not enough funding to do it all. Some prioritization is going to have to occur, and right now, that has been the SOC that has been tasked with that.

CHAIRMAN NORQUIST: Steve?

DR. LIPSTEIN: I just have one more question.

DR. WEISMAN: I just want to comment before you go to your next thing. I strongly disagree with you. They are advisory committees. They provide advice. The decision making body is the Board of Governors. They use all the information, including their collective experience, wisdom, and so forth.

I would imagine they take very seriously the advice from all the groups you mentioned, but the final judgment should come from the Board, including the fact they have heard the advice, they may elect to not follow the advice. I think that is the role of a board.

DR. LIPSTEIN: My question is being familiar with the statute, it says under the criteria that we're not funding questions of biological mechanisms. I guess where that question comes up in my mind is earlier today we talked about one of our pragmatic studies that has to do

with -- Gray, you and I have talked about this -- this is the lifestyle intervention with Metformin therapy.

The antipsychotic drugs have a biological mechanism that produces an undesired side effect. How do we not know it wouldn't be a better use of research money to study how to remove those biological mechanisms that produce the undesired side effect than it is to do the outcomes research here that we are doing?

CHAIRMAN NORQUIST: That would be NIH's issue, I would say, and Drug Development to come up with better interventions at this point. Until then, which is not likely to be in the near future, quite honestly, because it has been going on for quite a while, we will have to deal with, like in many other things we have in medicine, taking care of the conditions that are in front of us, whether they are caused by our own interventions or by something else.

Rick, did you want to make a point here? I'd like to get to that cutoff point there, to get

to the final.

DR. KUNTZ: Very quickly. The last point about because there is not enough money, we need to prioritize, and clearly there are finite resources, and we clearly need to prioritize for the targeted PFAs.

The conversation we had earlier to start the day about the pragmatic clinical studies program didn't suggest to me that we need to prioritize, it suggested to me to figure out how to solicit more and stronger and better applications.

CHAIRMAN NORQUIST: It may be in a year or two we may run out. I think you are right. At this particular point in time, we need more in, but we could be at a point where we do have to.

Christine, what's next?

DR. GOERTZ: You have all seen this. None of this is new.

CHAIRMAN NORQUIST: This is what the advisory panel uses.

DR. GOERTZ: Yes. I won't spend a lot of time on this. I do want to spend just a second,

because this is our Tier 4 criteria, this is where we are really looking at whether something should become a targeted PFA or not. This is how we sort list 5 into list 6.

CHAIRMAN NORQUIST: Back to that branch endpoint; right? Where you end up in the final boxes.

DR. GOERTZ: Yes. Either list 6 or list 7. This is how that decision would be made. First of all, we are interested when it comes to targeted PFAs really in looking at either a specific question or a set of questions that has been identified about prevention, diagnostic, or treatment options, or system level interventions that are currently covered, and used in at least some settings, and some interest for one or preferably more than one key stakeholder group, and there is a strong assessment of potential to change practice.

Also, it is something that we are interested in enough that we would want to set aside funding and have that closer involvement in

the study that we have with our targeted funding announcements.

It also may potentially require higher levels of funding than the usual pragmatic clinical trial. Right now, our pragmatic trials are limited with some exceptions at $10 million, with some of our targeted PFAs have been -- for instance, I think we set aside $50 million for the set of trials for Hepatitis C.

To the extent that we have a specific question, something of really high priority to us, and we feel the answer to that question will make a difference, not that we don't think the other answers will make a difference, but --

CHAIRMAN NORQUIST: Who applies these criteria to make that decision?

DR. GOERTZ: It is the SOC, but I would say as with all of these, we keep talking about the SOC does this and this, but it really is in conjunction with staff at every level. Staff would be presenting their recommendations to us about these.

CHAIRMAN NORQUIST: One thing I would just add -- did you have another slide or something?

DR. GOERTZ: There it is again, you can't see this too many times.

[Laughter.]

CHAIRMAN NORQUIST: Is that it?

DR. GOERTZ: I think that's it.

CHAIRMAN NORQUIST: Okay. Ultimately, the full Board does the green flags or whatever they are at the bottom there, the approval.

DR. GOERTZ: That's exactly right.

CHAIRMAN NORQUIST: If you go back to the other criteria, the only thing I would say is as you think about what should be targeted, each one of those bullets is not necessarily weighted the same because the key issue, what we were talking about this morning, bullet number two actually is probably the most important. You would weight that the highest, about whether you make something a targeted, so if something is really important, we really need to get it out there, it gets back to that issue, that's why it may need to be targeted,

and we may have to go out and contract with someone to get that done.

DR. GOERTZ: Right.

CHAIRMAN NORQUIST: When you think about these criteria, I would just say think about how you weight these and really focus on that second one, because don't wait for the pragmatic, which are only what, two times a year or something, if we really feel strongly about it, a hot topic right now, that needs to be a focus, and the Board should say go do that.

DR. GOERTZ: That's a good point.

CHAIRMAN NORQUIST: Others want to make comments?

[No response.]

CHAIRMAN NORQUIST: Have we burned everybody out now? I thought this was very helpful. Thank you. I think we need to be very clear publicly about what our criteria are, how we are getting to these, where we get at the endpoint, and I think that's good. It's not always transparent at a lot of other funding agencies.

DR. GOERTZ: Right. We are planning to publish these lists on our website so investigators have the opportunity to go and look at what we are currently considering, what is in the pipeline.

CHAIRMAN NORQUIST: Good. That would be helpful for people to know what's on the list. Ellen?

DR. SIGAL: All this is fine. I still don't know how we get to be more proactive, going out to the right people to answer them. It's still rather passive to me. The bigger question is there are huge gaps in practice in every single field today. How are we going to correct that. That, I still don't know. I understand this, but I don't understand how we get the hot topics and how we go directly to investigators who can really answer these questions.

DR. SELBY: That is the $64 million question. We spent an amazing amount of time talking to stakeholder groups. We solicited through the American College of Physicians, some very large number of clinical specialty groups, to

submit their questions. A number did. I would say maybe a dozen, clinical specialty groups submitted questions. We go to payers. We go to purchasers. We go to patients. Primary care.

Any new ideas about who to go to to get the questions are always welcome. You are absolutely right, we don't seem to necessarily be getting the exact right questions handed to us, even though we do put a lot of effort into it.

DR. SIGAL: There are these gaps. Again, it's anecdotal, but based on my own experience, when we go general, we get nothing. When we go specific, to the disease, specific groups, you can then get the gaps that are truly related. I don't think we have actually been doing that. I think we have been going very broadly to broad stakeholders, which is good, which is what we are supposed to do, but there are so many gaps in treatment every single day, and I don't think we are going to get it by going broad.

CHAIRMAN NORQUIST: That is one thing, making sure the topics that are coming in are

coming in in a way that we are getting it more specific. I thought you were also pointing out after we get the green approval and stuff, how do we get it done, which to me, and I think what Harlan was talking about and what my position would be, is a really very focused contract, this is the question we really want answered, let's put it out there, let's not just wait.

DR. LEWIS-HALL: This is Freda.

CHAIRMAN NORQUIST: Hang on, Freda, just one second.

DR. SIGAL: Two years ago, Joe, when I talked about process, I think it's the only way we are really going to do that.

CHAIRMAN NORQUIST: Freda, your turn.

DR. LEWIS-HALL: I agree that kind of specific questions, contracting them out, just really beginning to guide this more robustly is one way to do it. I fully endorse that as a way of advancing the agenda.

I also want to put a plug in for not just prizing some of the work but prizing for some of

the key questions. I may have shared this before. The Geoffrey Beene Foundation. We have been looking for years to try to get some ideas around the gender differences in Alzheimer's. Frankly, the work in this area has been impoverished. They offered a $50,000 prize, and got hundreds of applications with remarkable hypotheses on the difference, and kind of plans or recommendations or idea on how to study the differences as they were proposed.

It was really inexpensive. The answers came from all quarters, from mathematicians, et cetera, and may represent just another way to stimulate some ideas. You have to ask a specific question to get the hypotheses, but it still may be an interesting way to move the ball down the field.

CHAIRMAN NORQUIST: Thanks. Any other comments? I realize I've gone into the public comment period. Thank you, Christian, SOC, and the others who put a lot of work into this, and the staff, particularly.

DR. GOERTZ: I just want to thank the

staff and also the members of the SOC.

DR. SELBY: I thank the staff and thank the members of the SOC.

CHAIRMAN NORQUIST: We are all looking for some things in those little green things down there that we can get going. That is the key thing at the end of the day. The process is good but we need some stuff there. We need that green. Okay.

Sue? We are back into our public comment period.

MS. SHERIDAN: Thank you, Dr. Norquist. We have no one on the line that has registered to submit a public comment. I thank all of you who are still on the line but have not registered. If you have any comments that you do want to eventually submit to PCORI, go to INFO@PCORI.ORG, and we will shuffle that to the appropriate committees and individuals here.

If there is nothing else to share, then I think public comment is done.

CHAIRMAN NORQUIST: Thank you very much. Any final comments?

MS. HUNT: I didn't say this because I was concerned we really would run totally out of time at the end. I would really like to see -- I know we got rid of the evaluation group that was partly the Board, and it's gone to the staff, and I don't know what our evaluation plan is for the bigger questions of is PCORI meeting its goals, is it succeeding.

I know there are lots of little pieces. We have seen a couple of them today by Lori Frank, but I don't see the ones that are like really looking for overall, what are the outcomes of what PCORI is doing. I really would like to see evaluation of that, and I think it is tremendously important to the Board. It is part of our responsibility.

CHAIRMAN NORQUIST: Joe?

DR. SELBY: I think that is a great comment at this point, and I think we can go back to the strategic plan and look at those outputs and outcomes that are on there. We see things like publications mounting up now.

One thing we don't have a lot of is targets. We published 40 or 50 papers with PCORI funding. What was the goal? How many were we supposed to have published by now?

I think it would be good to go back to the strategic plan and see if there are not metrics right on the plan or outputs on the plan so we could begin getting that to you.

MS. HUNT: I'm not talking about counting things. I think we are doing a great job of counting how many articles we have gotten in peer review journals or how many clinicians have responded to surveys about have they ever heard of PCORI.

I'm talking about whether we have really been able to move the field forward in reaching patients, caregivers, and clinicians with information to help patients make decisions about their health care. That is what the bottom line is. I would really like to see outcome measures on that.

DR. SELBY: Yes. That is still a little

early by and large, and that is why those counts are in there for the time being. You have to write papers and get them published and things like that helps.

Again, we can go back and take a look and see if there may be things that are further down the line that look even more impressive, and I think we can really say that at the end of 2015/beginning of 2016, those CER studies will really begin, the final reports will begin coming in, the publications will begin.

The one example we have is one that I expect is going to impact practice, an editorial that a company said it would impact practice, so we are not too far from that.

DR. DOUMA: Joe, I agree with you that now that we have four or five years under our belt, it's a good time to look at the plan more as a plan versus a concept. Really hard to do seven years ago, but we have learned a lot, and we can do a better job of it now.

CHAIRMAN NORQUIST: Okay. Other comments?

DR. SELBY: I was just going to ask somebody maybe even more in the know than I am to say something about dinner at 7:00.

CHAIRMAN NORQUIST: Why don't we do that after we close and let the people go off line and stuff.

Let me close by thanking those who joined us today, and all materials presented to the Board today will be available on our website at PCORI.org. Today's webinar was recorded and will be posted by the end of the week. We always welcome your feedback at INFO@PCORI.ORG or our website.

Thanks again to everyone, and good evening.

[Whereupon, at 5:42 p.m., the meeting was adjourned.]