PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

BOARD OF GOVERNORS MEETING

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P R O C E E D I N G S

[10:22 a.m.]

CHAIRMAN NORQUIST: Thanks. Good morning. I'm Dr. Gray Norquist, Chair of the PCORI Board of Governors. I want to welcome everyone to today's Board meeting, which is being held in person here in Washington, D.C. as well as by teleconference and webinar.

Close to 200 people have signed up to join us online today and we're pleased to have you here. Instructions for logging in or calling in are available at our website at PCORI.org/events. All materials presented to the Board for consideration today will be available during the webinar and after will be posted on our website at PCORI.org.

The webinar is being recorded and an archive will be posted within a day or so. A reminder, there is a public comment period today as we always feature during our in-person Board meetings. It's scheduled for 5:15 eastern daylight time. We welcome comments both here in the meeting room and via telephone. Information on how to

provide a comment is available on our website at PCORI.org/events.

We welcome your feedback at other times as well, just email us at info@PCORI.org or provide input through our website at PCORI.org.

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

So, the first item is to approve the minutes from our last call, which was August the 26th, so I need -- those were sent out and I need a motion to approve the minutes, and a second. Second. Okay, thanks. Any discussion?

[No response.]

CHAIRMAN NORQUIST: All those in favor?

[Ayes.]

CHAIRMAN NORQUIST: Any opposed? Any abstaining?

[No response.]

CHAIRMAN NORQUIST: Okay, we are not -- because we're in-person we don't need to do a roll call since we can see all of us here. We would

just let the people on the webinar know that we have a quorum here, but before we get started, the first item was to have the Executive Director's Report, but unfortunately today is the last meeting for one of our esteemed members, Dr. Arnie Epstein, so we have to embarrass him here just a minute and we --

DR. EPSTEIN: We have to start this way?

CHAIRMAN NORQUIST: We have to start this way and we start with embarrassment. We might as well get it over with now, right?

So, we all have really appreciated, Arnie, what you've done. And for those of you who don't know, Arnie has joined, temporarily, I guess, -- just to make sure the Harvard folks know you're coming back, the federal government at HHS and because of that, it's kind of precluded him from being on our Board and we're sorry to lose Arnie, but we certainly have enjoyed your wisdom and we hope to have your input from outside when you're allowed to do that, but we want to thank you very much for all the work and stuff that you've done

for us.

It's really been very exciting. You were one of the originals, you know, it's hard to see you go at this point.

[Applause.]

CHAIRMAN NORQUIST: Joe, do you want to make any comments?

DR. SELBY: Sure, I'll take the opportunity to say something about Arnie. Arnie was notable, I think, on the Board for the parsimony with which he used the microphone and demanded airtime, unlike me and a number of my colleagues here, he spoke sparingly, but for that and other reasons, I think, when Arnie spoke, people listened. And behind the scenes Arnie was always -- I want to thank you for your patience, patience with me and patience with processes.

You have been a mentor and extraordinarily helpful, and the only reason that we are willing to let you go from this version of doing service to your country is that you found a much more intensive way to do it and so we're fortunate, we

thank you for that and for the four years you spent with us.

CHAIRMAN NORQUIST: And for all of that effort, we have this wonderful little gift for you, which is a nice little box with green wrapping and a blue bow on it. So, I hope there's something inside of it -- oh, in the PCORI colors. I missed that.

UNIDENTIFIED BOARD MEMBER: -- to $25.

CHAIRMAN NORQUIST: Oh, it was less than $25, by the way.

[Off microphone discussion.]

[Off microphone presentation.]

[Applause.]

CHAIRMAN NORQUIST: That was a standing ovation we were giving.

DR. EPSTEIN: That was a standing ovation and I'm touched and I'll say just a few words, keeping with parsimony. I'm one of a number of healthcare providers in our Board and I don't have to tell them especially how often they make decisions and I've made decisions, without having

the information I need to be confident that I was making the right decision, leave alone sometimes flying by the seat of my pants.

So, when I got a call about the opportunity to be on PCORI, to do the things we do and to try and bring information to bear where patients need it, I was delighted to go ahead and really do that. And despite the tone of the discussion this morning, which is we should focus more and choose where and do better, we do have a lot to be proud of and I'm really proud of for having participated in.

We stood up this organization. We got funding some important activities. We've created an infrastructure to start to support many more, and we're really, I think, poised to go ahead.

It surprised me a little bit that amongst the things I have enjoyed most about PCORI, though I care deeply about its mission and the work, has been my interactions with Joe, the staff, and the Board members, and I won't single people out individually except to point out the obvious.

Most of the time when I get to serve on a committee or a Board or a panel, it's because of expertise that I bring and it's usually accompanied by seeing the usual suspects, they're people who do the same thing I do more or less, a little of this, a little of that, and this is really unusually by legislative designation, we're sort of an ark and I remember being shocked when out of the 21 people on the board or whatever it was, I knew about four or five.

I guess I hadn't joined an organization like that, and it has been a distinct pleasure for me -- I'm sure many of you share this -- to meet the folks on the Board, to work with them. Each of whom has a different background, a different set of expertise, and a different take and when I think of the Board, what comes to mind for me is the old expression, no weak links, because every single person here comes here from a podium of accomplishment and expertise. And so that's been really great.

So, I will stand down after today. What

I'd like to remind you of, I'm reminded, Rick and I are the two big football fans here. I want to liken this to the second quarter because that's where you are. There's a game, and you're at about the second quarter, and it's a long way from over. And the decisions you make about where to stay the course, even though it hasn't paid off yet, or where to veer right and veer left, is what's going to make this a winning proposition or not.

And I'm betting on you. And I'll be watching from the sidelines, cheering you on, and I think you can do it. So, please move ahead with that vision.

Thank you very much, all of you.

[Applause.]

CHAIRMAN NORQUIST: Thanks, Arnie. So, now we move on to the Executive Director's Report. So, Joe?

DR. SELBY: Thank you, Gray, and good morning to everyone. I want to be the first to wish you a happy fourth birthday. In just about a week it'll be four years since that September 23rd

morning when the GAO announced the Board members and a couple months later you met for the first time, and the message for today, I think, is one of looking back at the accomplishments, having really established a firm infrastructure, a distinctive method of soliciting, reviewing, and funding research, a portfolio, which speaks to that, and preparation for moving forward from this point.

So, I hope that you can see this. It's a little tough with the screen so far away, but a timeline since the first Board meeting in November of 2010, this Board -- and really it was Board effort almost exclusively, got the pilot awards funding announcement released in September of 2011. Eight months later in June of 2012, the pilot projects were awarded.

In May of 2012, just after releasing our National Priorities and the Research Agenda, we released the first announcements for genuine patient-centered comparative effectiveness research, the so-called broad announcement, and those first awards were released just after the

methodology standards were adopted in November of 2012, so those first awards were given out in December of 2012.

Early in 2013, we released the first targeted awards in June of 2013, and by the end of 2013 in December, we funded the first targeted award, which focused on improving care and outcomes in asthma in minority populations. And in early 2014, we released the first pragmatic studies announcement and the first pragmatic clinical studies coming from that announcement will be awarded in January or February of 2015.

In the process, in these nearly four years now, we've elaborated some principles that have made their way into the research solicitations, the funding announcements, the application, the review process, and the conduct of our research, that is, that our research must be patient-centered, it has to focus on comparisons that actually matter to patients, their caregivers, their clinicians, and it has to consider the outcomes that patients say are important to them.

So, that's patient-centered. It has to be engaged. We don't do research without engagement of patients and other relevant stakeholders. We believe that involving key stakeholders from the beginning of the process makes our research more relevant and enhances the potential for dissemination.

And the third somewhat elusive but critical principle is that this research has to be likely to change practice. We are not about trying to understand mechanisms of disease. We are the last link in the chain. We are aiming for studies that can change practice, and this is challenging, it's unusual, I'd say, almost unique among funding agencies, but it's our mandate.

Okay, so we have now funded over -- if we do not count the PCORnet infrastructure investment, we've funded over $400 million -- well over $400 million in research, almost all of that from the broad announcements. Key point is that in each of our priority areas we have seen the emergence of themes with multiple projects, so if you see a

theme up here, it's represented by five or more, in some cases more than 20 projects.

I think you will agree that these themes speak patient-centeredness, these themes speak a different approach to funding, a different set of topics. This is our portfolio. It's the result of our shared work, the Board, the Methodology Committee and the staff, we are now working hard to monitor and manage these projects and to synthesize the learnings from these themes.

So, I'll just go through them quickly. In assessing prevention, diagnosis, and treatment options, self-care, caregiver support, palliative care, under improving health systems, care transitions, the role of telemedicine, the role of patient navigators in coordinating care, collaborative care, that is bringing behavioral and physical health together in the same settings.

Under addressing disparities; tailoring interventions with cultural and language training, the role of community health workers, the role of self-management, and under communication,

dissemination research, shared decision making and parental support in pediatric illness. These are themes that are distinctive, they are -- they have become widely known across the country and these kind of themes are associated with PCORI and over the next year or two we will be reporting back on the results of these studies and we hope that the Board holds us to this, to report back to you on what we've learned from these important and unique theme areas.

But I also want to point out that these four years have been a refining and evolving process of increasing specificity, focus, and larger expenditures. So, the pilots were up to $500,000 for two years and they explicitly were not comparative effectiveness. They were research projects on how you do research while engaging patients and other key stakeholders. There were no restrictions on the clinical area.

Then we got to the first CER, which was a broad announcement. These emphasized patient-centeredness, stakeholder engagement; they had to

be comparative effectiveness research, but again, no stipulation about the clinical area. Any area was eligible to be considered for funding as long as it offered an opportunity to change practice. These were somewhat larger, up to $1.5 million in direct costs, up to three years in length.

Then we moved to the targeteds. These could be -- these were for much larger amounts of money, in fact, the fall prevention initiative, as you know, was a $30 million investment. They also are always comparative effectiveness research that involves patient-centeredness and more robust engagement is expected and they are focused on single clinical areas with narrower research questions.

And finally, the pragmatic clinical studies are CER, they require head-to-head comparisons of specific questions. We put out the high priority topics, we review the letters of intent for specific questions, they are large, they can be up to $10 million in direct costs and last five years. And they're associated, as I said,

with a set of high priority topics, which comes from our advisory panel, so, the stakeholder driven infrastructure we've built for getting to high priority topics.

So, a trajectory that's gone from completely non-targeted to rather specifically focused in the pragmatic studies.

Here are the topics in the targeted PFAs, and you'll see that each one of these is a specific disease and with maybe one exception and that is transitional care. Three of these have been funded or I should say the first two have been funded, the third one, the treatment options for uterine fibroids is about to be announced just about any day, is my understanding. Transitional care and obesity, my understanding is we will bring these proposed awards to you in our last meeting of this fiscal year in late September.

And the improving blood pressure control in vulnerable populations has been approved by the Board and is being developed in collaboration with NHLBI now. So, those are our targeted

announcements. You see they've gotten more specific.

I'm not going to scare you -- don't worry that I'm going to try to take you through this again, but just to say that we have worked diligently with the Scientific Oversight Committee at refining our prioritization process. The key points are that we involve the SOC and we're moving toward involving the SOC earlier in the process.

The advisory panels, the multi-stakeholder advisory panels play a key role in prioritizing topics and they can come out with one of four recommendations, one is to not pursue the topic, one is to include it as a mention in the broad funding announcements, the next is to include it as a high priority topic in the pragmatic clinical studies announcement, and the most intense would be to issue a targeted funding announcement on this topic.

We continue to work on refining this process with the SOC, but I think we've got the ingredients here and it's a matter of making this

work efficiently and quickly.

So, we're very excited, particularly on the staff, at the prospects for the first set of announcements, awards, under the pragmatic clinical studies announcement, so this slide and the next show the topics that have actually been submitted to PCORI, the final proposals that have been submitted.

So, you'll see on their treatment alternatives for bipolar disorder, the management of ductal carcinoma in situ, breast cancer screenings -- alternative approaches to breast cancer screening, the use of colony stimulating factor in cancer, just a couple more, alternatives for venous thrombosis prevention, genetic testing for coronary artery disease, new therapies -- comparative effectiveness of new therapies for hepatitis C, the use of proton beam therapy versus traditional radio therapy in cancer, new therapies for sickle cell disease.

So, these are high priority topics. I think unlike the state today when you look at our

portfolio, in January you will see at least six of these topics in our portfolio with a sizable investment of funds. That changes -- begins to change the tone of the way we think about our portfolio and gives us a lot of opportunities to build on the studies that we fund through this initiative.

Here are some patient-centered versions of these very same questions with regard to hip fracture. Just take a look at those, I won't read them off here. People at home are listening and can also see the slide, but just to say that these reflect questions that face patients and their clinicians every day. And those are the kinds of big studies that we will be funding.

So, as we move toward 2015, we will build on the foundation of patient-centeredness, stakeholder engagement, and research that's likely to change practice. We'll actively manage the portfolio that we've got, synthesizing knowledge from the thematic areas I showed you and identifying opportunities for dissemination from

this working partnership with AHRQ.

We'll work -- we are working with the Science Oversight Committee, with our advisory panels, with our stakeholder groups, with those who write our topic briefs, in fact, to continue moving toward -- more swiftly towards highly specific focused high priority questions. This is really, in my experience, the most surprisingly difficult challenge is to get to what's the right question, and as someone said a little earlier today, you can get even a group of experts in the room and still come out with five different themes on what's the exact next topic that will change practice, but we continue working on it and we are getting closer.

And we will identify methods, along with the SOC, to move these topics through our topic generation and prioritization processes more quickly.

Okay, so that's just a few comments on where we are. Some late-breaking news now, we have gone through a good part of the first GAO review. We received our notification in March and they said

that the two topics they really wanted to focus on most closely at this point in our history were to what extent have we established the research priorities and the funded research in accordance with the legislation, and to what extent are we making efforts to evaluate the effectiveness of our work.

We had an entrance conference in April, we've had a number of meetings with GAO team, each focusing on a specific topic such as the role of our advisory panels, how we plan to do dissemination, how we're doing evaluation, our merit review process, PCORnet, and research funding. There will be some additional meetings in late September, early October. We expect an exit conference with the GAO at some point during the winter, probably in December, and then GAO's report will be delivered to Congress in March of 2015. So, we will keep you posted on this process.

I just want to say that in case you have not seen it, in case you haven't been to the PCORI website over the weekend, we have a new website.

So, after about 11 months of very hard work that involved a consultation with a large number of you, a lot of stakeholders and staff, we have a new really second decade of the 21st century website that is prepared -- you can't say 21st century because the first one was that too, but this one is really state of the art. It has greater flexibility, it serves users much better from patients to applicants, it's really expandable because we know we're going to be using it for all manner of activities going forward.

It uses more video, more interactive features, and very importantly, don't do it during this meeting but when you have a break, get out your iPhone and go to www.PCORI.org on your iPhone and you'll see that it's perfectly fit to your iPhone now, so that's -- Bill Silberg is real proud of that. So are we all.

And somehow my changer just stopped working, my advancer. It works just as well on a Droid phone, I trust.

We don't have an app, but we are working

on an app, right, Orlando? Yes.

Okay. Here's a big -- I think this is my last piece of news, but this is a big piece of news. Sharon Levin, attention, we're going to have an annual meeting, our first annual meeting in the Fall of 2015. One of the -- it'll be an opportunity to update our work, particularly on some of those thematic areas I showed you. It'll be multi-day, multi-track. A lot of our awardees will be there, but we will also have plenary and panel discussions, invite some research patient partnerships to present, hold some how-to workshops and evidence, action, network sessions.

So, our engagement folks, our science folks, probably our contracting folks will all be heavily involved, and our program planning committee has been formed, is my understanding, so we're talking about it and we will update you more on this quickly.

So, for today, in just a minute you're going to hear about the dashboard, get your next dashboard update. Then we have a discussion,

really a presentation on the 2015 budget and hopefully a vote for approval. Then you're going to hear a second iteration on the peer review and release of research findings draft proposal, which we're going to ask the Board to approve for postings for public comments. So, this is the last item in the legislation that we have to post for public comment. We will probably post other items, but this is the last one we're mandated to. And we're very anxious to get that out.

You will hear a presentation over the noon hour from four PIs within PCORnet, two from CDRNs and to PIs from PPRNs with a panel discussion that will be a great chance for the entire Board to catch up a bit on how PCORnet is progressing after six months.

We'll hear a Methodology Committee update and you'll be amazed at the amount of activity that the Methodology Committee has embraced and engaged, and then you'll hear, as Gray mentioned, two very interesting presentations that will be presented back-to-back, one on improving healthcare systems

presented by Dr. Steve Clauser, the program director for our Improving Healthcare Systems program at PCORI, and then Dr. Rick Kronick from AHRQ will present the portfolio funded by the PCORI PCOR trust fund at AHRQ. And it will be very interesting, I think, to put those two back-to-back and consider how we complement each other and avoid overlap and make sense of our dual but kind of closely related missions in this area.

So, Gray, I think there might be time for a question or two if -- how are we doing on time?

CHAIRMAN NORQUIST: [Off microphone.]

DR. SELBY: So, we've got -- still have about 25 minutes.

CHAIRMAN NORQUIST: Why don't we take any questions that -- on what Joe has presented so far and then he needs to do his dashboard review. Harlan Weisman. By the way, since we're not on camera, will you please say your name.

DR. WEISMAN: Harlan Weisman. Joe, thanks for the overview. You listed two points of interest of the GAO, the first one on

prioritization I understand and I know we have an effort underway for evaluation of effectiveness of PCORI. Can you -- when will we know more about that, and, one, the process of doing the evaluation, our self-evaluation, and two, the results of that evaluation?

DR. SELBY: Good. There's actually an amazing amount of work going on in evaluation. I was just saying to Michele Orza that it's really time for a Board presentation. There is an external PCORI evaluation group with external evaluation experts as well as Board and Methodology Committee members, and there's a lot of work -- we've really been spending a lot of effort getting our data into order and beginning to evaluate more aspects of our portfolio, the investigator pool, the merit review process. So, we will aim to get a presentation either on an open Board call or at the November meeting to update you on evaluation, a lot to be said there.

DR. WEISMAN: Thank you.

CHAIRMAN NORQUIST: Ellen.

MS. SIGAL: Thank you for the presentation.

CHAIRMAN NORQUIST: Wait, you need to say your name.

MS. SIGAL: Oh, I'm sorry. Ellen Sigal, Board member. Thank you for the presentation. I just want a clarification on the dashboard. So, the topics for focus going forward are incredibly important. I just want to make sure they're not comprehensive and there are others that may be added to it.

DR. SELBY: On the dashboard?

MS. SIGAL: Yeah, on the priority setting -- on the focused priorities that you listed, the two pages.

DR. SELBY: Yes, I think the topic -- one way or the other, the topic prioritization process continues and I think we will always want to be open to new and important topics as they arise, so, yes. New topics will be added. Some may well be taken off as well for one reason or another.

CHAIRMAN NORQUIST: Allen.

DR. DOUMA: Allen Douma, Board member.

It's exciting to see, as you're presenting more and more data as we go on and celebrating our fourth anniversary, that there's research that was funded a couple years ago and we actually have some results that are out. I would just like to urge us to make it more easily -- make it easier for people to find the results of our research and we might even want to -- for example, our database that you can search on the new website has the date something was funded but not the date it's expected to be completed, and that would be helpful.

And also consider -- we did put a lot of work into developing topic briefs, for example, which could be excellent information for a lot of people as a summary of what's going on out there and so consider that as product of our work as well.

DR. SELBY: Good. I believe we do post -- we do post the topic briefs. Is that right, Romana? I know I've seen some. So, you can find the topic briefs from our advisory panels on the website, Allen, and I will mention that two of

them, one on ductal carcinoma in situ and I think the other one, treatment of bipolar disorder actually were published in the same issue of the Annals of Internal Medicine.

DR. DOUMA: Yeah, I apologize. Perhaps we could make it even easier for somebody like me to find them as a separate --

DR. SELBY: With the new flexible website, that should be a cinch.

CHAIRMAN NORQUIST: I don't see any other cards up. Okay, Joe, you're on for the dashboard.

DR. SELBY: I promise this is almost the last thing I'm going to be presenting today.

Okay, so here we have a picture of three dashboards, and this is all about -- and you actually have a copy -- no, you have a copy of the dashboard in front of you, but this slide is a way to explain that dashboard. You can see the three colors in the upper left, green means we're on target, the yellow means we're off-target but we've got an explanation and we think it's -- you know, that it's under control and we are going to make

it, ultimately. The third is that we need to talk about this, it needs attention.

In the lower left you see a version of a dashboard, which is not ours, that is entirely green. In the middle and the top row, you see a dashboard, which, in fact, is ours. It has a lot of green and it has a lot of yellow, it has no red, and then you see one to the right and the lower corner, and that also is not ours that one would mean there was a lot of significant problems to discuss.

So, we're talking about the dashboard that looks like this, and I'm going to highlight -- I'm not going to talk about everything, but I'm going to highlight some of the areas both on the green, because it's important to celebrate, and on the yellow.

So, the first thing I'm going to talk about, we will always have on the dashboard a kind of qualitative story about some way in which we're either growing our research or disseminating and implementing it or influencing research conducted

by others. We're also going to talk about the impact of engagement, of involving patients and stakeholders in the merit review process. We're going to talk about the ambassadors, the PCORI ambassadors and training for those, and we're going to have a happy little story about responsiveness of the research portion of PCORI to the inquiries of would-be applicants. So, we're going to go to those first.

So, this is just a story, I won't read it except to say that Dr. Debra Fiser, after many years of being the Chair of Pediatrics and then the Dean of the School of Medicine at the University of Arkansas, as she was winding down -- really, I think she was dean for something like -- oh, seven years it says -- she went to a PCORI town hall meeting in Memphis and decided after that that what she would do as she went back to mentoring and training young physicians was to set up at the University of Arkansas a Center for Comparative Effectiveness Research in Pediatrics. And my understanding is it was funded by UAMS. I visited

its website over the weekend and they are training young investigators to engage patients and stakeholders and conduct comparative effectiveness research. So, that's just a nice example, one of many, really, we hear about, of influence that PCORI is having on the research community and partnerships between researchers and other stakeholders around the country.

So, this is the result of a merit review survey. So, we survey our merit reviewers, the patients, the other stakeholders, and the scientific stakeholders, after every cycle, and this shows you vertically the first -- or, from cycle two through the winner of 2014, so the four out of the first five cycles, skipping the first one, and you see the sample size, you see the response rates, I guess it fell off just a little bit this last time, but we get very high response rates, and the percent who agree somewhat or strongly with the statement the patient stakeholder reviewers provided valuable input during the discussion is high.

In fact, if anything, going up recently; so from 85 to 94 percent and we broke this out by the type of reviewer.

So, you won't be surprised that the patients were quite convinced that they provided important input. They are the violet or purple on the left. The other stakeholders, just about as convinced that they provide meaningful input, but even the scientists, who were the ones you might suspect would doubt whether those non-scientists were contributing, upwards of 80 percent of them always said that the patients and stakeholders provided valuable input during the discussion.

Here's a second question, "The scientific reviewers provided valuable input during the discussion", and they appeared to be doing a good job. All three groups scored their contributions as very important. And here's one, "Overall, scientific reviewers were receptive to input from patients and stakeholders." And, again, here you need to look at predominantly the patients and stakeholders, but they are very high on reporting

the extent to which the scientists took their perspectives into account, and I think -- you know, I credit our merit review team at PCORI for the training that they do in an ongoing way and in many ways, the mentors that support our patient and stakeholder reviewers so that they can hold their own in these discussions.

Also, in case you haven't seen it, I wanted to draw your attention that was in the Annals of Internal Medicine, which basically says, and this is from our first cycle -- which essentially says that patient and stakeholder reviewers and scientific reviewers disagree with each other dramatically after they've read and scored the applications and before they've had the conversation. After they come together and have the conversation, they come dramatically together so that the scores of patient stakeholders and scientists are very close, after discussion, and that everybody has moved.

It's not that only the patients have moved, so this is in some ways our first

quantitative research publication and we are currently following it up, because there's a lot more to be asked, but this analysis of merit review scores is very useful and it has not been done very often by anybody. And I will -- I want to say a special -- acknowledge Rachael Fleurence for leading this work and really thank Mike Lauer for being a very steady thought partner and co-author on this.

But as I said, this research continues and we will have subsequent papers telling you more details about how these score patterns trend.

This is the ambassador program, a program in which stakeholders of all sorts are trained to represent PCORI, to speak about it, and to promote it, and you'll see here that the numbers are high and the groups include large numbers of patients, large numbers of advocates, that is, folks from patient organizations but also large numbers of researchers and clinicians. So, the community of people around the country has grown and I believe this is actually ahead of the numbers we planned

for this year.

This is the story about science response time. Two pieces to this, the first time the blue line, first, I'll draw your attention to -- we had -- in quarter four of 2013, we had 7,800 tickets submitted, that is questions submitted by applicants. We revamped our applications and improved them dramatically and the number of tickets has gone down and been rather steady at about 4,000 to 5,000 since then.

But now I'll draw your attention to the lighter blue vertical bars, our ability to respond within three business days to inquiries has gone from 54 percent to 99 percent, and so now our science response times are equivalent to our contracts response times, and I have to call out one person here, this is Emma Djabali who is over against the wall, and Emma really has spearheaded this effort to enhance our response time. So, I think those of you who feel that questions about us not being responsive to phone inquiries or email inquiries, hopefully that will taper -- it should

taper off because we are getting back to people much more quickly now.

So, now I want to address some yellow flagged items, the first one is the percentage of projects that are meeting all milestones, the second is our Pipeline to Proposal Awards, which are going to be less than we anticipated by the end of 2014, the completion of Phase I of PCORnet, we're only a third of the way through it, but our estimation is we're not going to quite hit the milestones we've set for ourselves and our networks by the end of 2014, fiscal year 2014, which is the first third of Phase I.

So, this is the progress on our research projects and the percent of projects that are meeting all milestones, so the blue in the middle looks a little worrisome. I'll draw your attention first to this top row, which is just the number of projects on which we're reporting goes from 23 to 140, and by deduction you'll figure out that it means we're further into some of these projects in quarter three than we were in quarter one as well,

and the middle row suggests that the percent of projects meeting all milestones due is dropping from 67 percent down to 51 percent, but the bottom row is very important, the average percent of milestones due that were met has stayed exactly the same, at mid-80s.

So, this is familiar to any of you who do performance measurement, quality measurement. If you have one indicator, you may be at 80 percent, but if you say, how many -- and it may be at 80 percent for all three indicators, but if you say how many people are on target, meet all three, it necessarily goes down, and so that's what we're seeing. People have gone further into the projects, they have more milestones that have been evaluated, and therefore, this is going down, not to say that we don't need to address this and keep our eye on it, but it is to be expected and take some comfort in that bottom row.

Here's an idea of, again, on the left you see the proportion of meeting all milestones going down, and then you see some of the milestones we're

talking about. So, we have not incorporated recruitment milestones. We anticipate that we'll be able to begin telling you about recruitment milestones when quarter four data are in.

You see that the proportion who have obtained IRB approval on schedule has dropped a bit. It's still pretty good, because this is usually a quarter one requirement, part of that had to do with -- I believe -- with PCORnet as well.

And the next is the percentage of studies in which payment has been withheld for programmatic reasons because the study is not meeting key milestones and that is very low, but we'll keep an eye on that.

And the last is the contract modification process, so now in the third quarter, 4 percent of contracts have had to be modified to adjust for failure to meet milestones, and just in case you're wondering like I did, say, what are the other milestones -- every project has different milestones, so it's not like -- I can't tell you that there's this fixed milestone. Different

projects have different milestones and different numbers of milestones based on what the proposal is.

Okay, this is the Pipeline to Proposals award, and you remember I said that we're going to fund fewer of them. We funded 30 Tier I awards, that's the entry level, smallest and the beginning, early in fiscal year 2014. They're in fact, nearing completion. They're about pre-engagement and community building. They were up to $15,000. We have not funded yet any Tier II, which is about partnership and infrastructure development or Tier III, which is proposal development, yet.

The goal, in this entire process, is to drive toward PCORI research proposals submitted by teams that started with the Pipeline-to-Proposal award.

We don't anticipate making any more awards in 2014 and the case is actually that, as Jean Slutsky came aboard and joined the Engagement Team, there was a period of reexamination of these and I think they've come out revitalized and focused even

more sharply than they were.

So, our initial 30 awards and the first program office for the west are giving us data and feedback that are refining our approach. Twenty-nine out of the 30 projects have met 100 percent of their milestones. Early next year we will fund four additional regional program offices, so there will be five. And we anticipate up to 50 Tier I projects. We anticipate a number of these coming back for Tier II projects and up to 50 Tier III projects as well.

So, the Pipeline-to-Proposals program is alive and well. It's our approach to getting a broader cross-section.

And, lastly then, a progress on PCORnet. The point here is precisely that the one item, the one milestone that's lagging a bit is the governance policies. These are critically important. We've identified them as critically important. And we're working closely with the Governance Taskforce in PCORnet, so they will be coming and you will be hearing about them,

particularly the RTC will be reviewing these governance policies with the Governance Taskforce and PCORnet leadership.

Other than that, I think the other milestones at the PCORnet level, communications, steering committee, the clinical trial, the topic has been determined, and the common data model, are on target, are on pace.

So, with that I'll ask, first of all, do you have any questions about anything you see on this dashboard? And secondly, are there items that ought to be on this dashboard that aren't? Are there items that ought to come off the dashboard because they just aren't worth the real estate?

CHAIRMAN NORQUIST: Okay, thanks, Joe. Gail?

MS. HUNT: Gail Hunt, Board. Joe, do we have the results of any of the pilot projects? And where -- has any of that -- any of those results or indicated areas where we should be funding additional work, you know, things that came out to be really top notch? Because I know we had some

that were supposed to be due by now.

DR. SELBY: I think the results are coming in, but we have been managing them. I'm actually not prepared to give you the detail. I know that there are about five key clusters of topics. I believe some of those investigators are working together on identifying next steps. I think one of the reasons we want to get the peer review and release of research findings policy out for public comment is that we're going to have these reports coming in from the pilot projects beginning, as you say, just about any day and we're going to want to be able to post them in a timely way.

I think also that you'll hear a lot from the pilot projects at the annual meeting next year, in fact, I think there's also another meeting just for the pilot projects early in the year, if anybody hear who wants to back me up on that -- Jason? Jason is not here? Okay.

CHAIRMAN NORQUIST: We'll find out the answer. Yeah, Bob?

DR. ZWOLAK: Bob Zwolak, Board. Thanks,

Joe, very much. My question surrounds the milestones and the project milestones. What's our action plan for projects that aren't reaching their milestones? And how did you decide that our milestone bar would be yellow as opposed to green or red? Is our metric for meeting milestones based on other funding agencies? Did we decide that up front or is that a retrospective application of yellow?

DR. SELBY: I might ask Michele if she'd like to say how we decided that it was yellow. I think it's that we saw it going in the wrong direction. I don't -- to my knowledge; we have not set a target yet. It's actually a good suggestion that we do set a target to monitor ourselves over time. Michele, do you want to say anything about how we chose to make this yellow? Am I right that it was mostly going in the wrong direction?

MS. ORZA: [Off microphone.]

CHAIRMAN NORQUIST: Wait just a minute because they can't hear on the webinar. What she was saying is that the reason we put it in yellow

is because it was going in the wrong direction and it was to highlight it for discussion. I think because there actually is a clear set point here that we're trying to go --

DR. SELBY: I think you're thought that we ought to have a target really applies ultimately to a number of things on this dashboard and one way to identify a target is to talk with people like Mike Lauer and Rick Kronick about how they might do it, talk to other funders about how they monitor this.

I think we have a lot of data and increasing amounts of data that we can look at in this area and I think it's a critical area if we want to get timely research.

CHAIRMAN NORQUIST: This is Gray. Let me just add on that having spent 15 years doing this and when I was at NIH and we got to a point where what happens, particularly in trials in your recruitment, you keep pouring money in hoping they're going to get more and at some point you have to say we cut our losses and move on. And what we did is, before I left, we just made a

decision. You had up to -- and I don't remember the exact figure but it was something like a year and a half to get 50 percent of the recruitment or something and we had some kind of way of doing it. If you didn't make it, we'd just stop the study and we took the money the next time and used it for another study, and we don't have such a policy and I think we need to do that because otherwise you can fall into this trap of going, ah, we're already 50 percent into it. We shouldn't just keep pouring money into it. And at some point you have to be able to say, cut your losses and -- but you're very clear with the investigators up front, this is what we expect, so you don't surprise them one day and say, we're just cutting you off now.

So, maybe Michael and Rick want to just say something.

DR. LAUER: Yeah, a couple things. One is that at NHLBI we actually have a published recruitment policy, which we tell investigators that they have to look at before they even submit their application, and it defines rather

specifically green, amber, and red zones and what will happen if they fall into those areas.

The second is that for some of our projects, we fund -- particularly trials -- we fund them in two phases, we call them in our parlance U2 and U3, and essentially what that means is is that the first phase is get your ducks all lined up in a row and show us you can actually get this thing going, and we have an understanding that if, at the end of this phase, you haven't achieved those milestones, we stop the project without having to have a long drawn out battle. And if you have demonstrated to us that you can do it, then we press the green light to move forward.

CHAIRMAN NORQUIST: Yeah, so I think we need to make sure we have some kind of policy like that and that we publicize that so it's very clear. Any other questions -- oh, I'm sorry, Allen.

DR. DOUMA: It's Allen Douma. Just want to reinforce what you just said, Gray, and excuse me, Joe, typically a dashboard -- anything that parameters on a dashboard does have something

you're shooting for, and there may be reasons that you've got to make a really big guess, but it still shouldn't be guessed at a priori, otherwise you can't measure against anything. So, I think it's great that what you just said is true.

There are a couple of items here where the numbers that are provided look like they're Q1 numbers and clearly it would be helpful if those could be updated sometime in the near future, the one on the bottom left you've got research funding commitments -- excuse me, expenditures is between Q2 and Q3, other 2014 expenditures, it looks like -- I'm presuming what that means is that number there was something that a --

DR. SELBY: Yeah, thank you very much, Allen. These arrows are tricky to look at and what the number in the circle means is the amount we had spent at the end of the third quarter, so we're --

DR. DOUMA: Oh, okay.

DR. SELBY: So, we're showing that this is the end of the third quarter dashboard and those numbers -- it's just that we've spent where we

anticipated spending in one quarter --

DR. DOUMA: Okay. And that's assuming that your spend would be even across the entire timeframe? It would have been?

DR. SELBY: No, I don't think that's true, I think we actually did spend -- it looks like it on the -- I agree with you, it looks like it on this dashboard though, on this diagram, they're not exactly maybe where we planned, but --

DR. DOUMA: Okay, but the placement of those numbers in the circle are not relevant to when the number was generated?

DR. SELBY: Right, they're the third quarter or whatever quarter the dashboard --

DR. DOUMA: Thank you, Joe.

CHAIRMAN NORQUIST: Other? Yeah, Ellen?

MS. SIGAL: Ellen Sigal, Board. I wanted to reinforce this issue of metrics on accrual and I would say that it's been a major change of the NIH now in institute-by-institute has their own policy. I think we have to do better than that. I think accrual is slow and if we're going to do this, fund

these trials, we should make sure there is a rigorous plan up front and our metrics, I would argue, should be higher than what they have at NIH because we have less burden and regulatory issues. So, we have to really look at that and set a very high bar.

CHAIRMAN NORQUIST: Thanks. Any other? Joe, one thing I would say that dawned on me, when I looked at this responsiveness to contact, I think one of the key issues is the satisfaction with their responsiveness, because you may want to think about rechanging that. I mean, the fact that someone responds within three days is great, but it's the type of response you're getting.

So, some of the concern I hear is that -- back from some folks, I mean, this is just a sample of one here, but myself, what I'm hearing from people is, yeah, but I'm not getting the kind of feedback that I need, and so I would propose that you change that particular metric to more of a satisfaction metric now for the potential grantees and stuff so we get a better idea about the quality

of the responses.

DR. SELBY: Good. That's a very nice evolution.

CHAIRMAN NORQUIST: Okay. Well, we're almost on time then, we picked it back up, so we're five minutes into the budget. So, now we go to the Consider for Approval, the FY 2015 Budget and I think Regina Yan, who's our chief operating officer, and Joe, will do this, but Regina's going to -- where is Regina? Oh, there she is. And Kerry Barnett as head of FAC may -- I mean, you guys oversee this, so if you have to, you can have some comments on this too.

And we did have a discussion about the budget a little bit, but -- the other week, but this is formal discussion in public.

DR. SELBY: So, this budget is the product of a process which began three to four months ago that involved discussions with each strategy committee, particularly of the planned expenditures in the area which that strategy committee oversees. It also involved several iterations with the FAC,

and Regina and I thank the FAC for their patience and for their input in getting the budget to the point it's at now.

This has felt like the most thoughtful process, budgeting process to date. I've been around for, I guess four of them now, and this is the -- has really been a much more integrated process, not to say that it won't be even better next year, but I think with that I'll thank Regina for the amazing amount of work that she put into it and work that a number of people on Regina's staff contributed to this, and I'm going to sit up here and maybe field questions along with Regina after her presentation.

MS. YAN: Thank you, Joe. In today's presentation, we will review PCORI's cash situation and our financial position overall, and also review our proposed 2015 budget.

I thought we'll start with, again, clarifying some key definitions of a couple important terms, particularly regarding commitment. When we talk about commitment we're really talking

about the funding -- amount of funding PCORI intends to award or the funding that we have already awarded in the past couple years, and these will be the multi-year contracts that we have executed and awarded for our research projects, our infrastructure, which is PCORnet, and also our engagement awards.

So, commitment, we'll be talking about the funding we plan to award. And then the other one is the expenses. Expenses is going to be what you will see in our budget, particularly when it comes to the program portion of our budget. That will reflect the actual expenses that we see as reported back to us through invoices from our awardees for all the contracts that we have awarded, including for research, infrastructure, which is PCORnet, and also the engagement awards.

So, for a new organization like us, we're only four years old, we see a lot of commitment early on with expenses trailing a little bit behind, but we are now, this year, beginning to see expenses picking up the pace. First is our cash

flow. We are just two weeks away from the end of our fiscal year and we expect our cash balance at the end of this fiscal year to be $667 million and our cash receipts for 2015 at $469 million, that includes both the appropriations, the transfer, as well as the PCOR fee, and then the cash disbursement for 2015 to be at $362 million.

Our cash balance at the end of fiscal year 2015, we will be looking at $774 million. The $774 million is not idle cash sitting around and you think that maybe we should put it to use. Actually, this is going to be the funds that have been fully obligated for all of the awards that we are going to -- all the projects that we are funding.

And the cash balance reflects both funds we have in the PCOR trust fund and also the funds we have in our bank account at Bank of America.

If we look at the funding commitment that we have made for the last couple years, for 2012 and ’13, the funding commitment we’ve made primarily in the research area is $331 million and

for 2014 we are looking at commitment level of about $400 million, half of it has already been approved by the Board, and we have one more round coming up in two weeks and there will be another Board meeting that the Board will be asked to approve another slate of awards. And for 2015, again, this is a funding commitment plan, this is a plan that we've put together and obviously like any of our funding announcements, all of them require Board approval, so these may change, it depends on Board's final approval, on both the topic announcement as well as the final award.

Our awards are in three major categories, research awards for 2015, our plan is for $475 million, for infrastructure, which is PCORnet, it's $265, engagement and Pipeline-to-Proposal awards, $21 million. So, for 2015, our funding commitment plan will be at $661 million.

So, last year the Board approved a funding commitment plan of two years at $1 million -- $1 billion, sorry -- so, if you look at '14 and '15 we are looking at about $1 billion level. And so by

the end of 2015 we'll be looking at cumulative funding commitment at about $1.4 billion, out of which 80 percent will be in research, 19 percent in infrastructure, and 2 percent in engagement awards. That is the mix so far, but of course for infrastructure currently we're only look at 2014 and 2015 that will be part of our commitment plan.

As a funding organization, we do need to plan way ahead in looking at our funding plan. This is a funding plan that we've put together through 2019. So, through 2019 we are now looking at 2019 as the last year we will still have funding awards at about $200 million and right now we're looking at 2015 as $661 and 2016 will be $400, $300, and $200.

So, if we look at our plan through 2019, of course, again, you know, the final decision is pending the Board's decision, obviously, and your approval throughout the year as we look at what we plan to fund.

So, at the end of 2019 we'll be looking at funding level at $2.6 billion out of which 87

percent will be in research projects, research contracts, 10 percent in infrastructure, which is PCORnet, and 3 percent in engagement awards, which include both the Eugene Washington Engagement Award and Pipeline-to-Proposal Award, so that is the overall mix. That's what we're looking at this moment.

So, the next one we'll be looking at both commitment and expenses, which if we look at the funding commitment level we planned for from now through the end of 2019, what is the expenditure that we're looking at throughout that period? So, as expected, early on most of our work is focusing on the commitments and our expenditure is very low, from 2012, '13 to '14, and then 2015 you'll see that the expenditure level is picking up and after 2019 awards, we will see the expenditure, that means these are the multiple year projects, we see expenditure coming in all the way through 2021 and a little bit in 2022.

What that means is we will have to have our staff plan and everything ready to support all

the awards that we have made in 2019 all the way through 2022.

This is our proposed 2015 budget. This year we have multiple opportunities for our committees and the staff to look at our preliminary draft budgets so that we can incorporate your feedback in the final presentation, so we thank all of you for your contribution and feedback throughout this process, and I think that with all the committee review, it has really improved our process to make sure that the details and the presentation is in the format that is easily understood by everyone.

So, for 2014, we are close to the end of fiscal year now, our expenditure is looking at about $126 million with $81 in program expense, which breaks down to research expense, infrastructure, and also our engagement awards. These are the three big buckets of our programs, and then with program support expense at $27 million. I will talk a little bit more about what program support expenses are, and administrative

support expense at $17 million, so that breaks down to 64 percent in program expense, 22 percent in program support, and 14 percent in administrative support.

And for 2015, with our proposed budget, we're proposing a $361 million with $271 in program expense, which it means actually expenses coming from the awards that we have made -- pretty much already made by early next year; about $60 million in program support and $30 million in administrative support. That breaks down to 75 percent in program expense, 17 percent in program support, and 8 percent in administrative support.

As we expect over time, our program expense will pick up because of all the awards that we have made, commitments that we have made, so we do expect that our administrative ratio will trend down because of that, and I know that some people will consider both program support and administrative support as in one big bucket. For those people who like to see it that way, we have broken down the percentage by each category, so you

can look at it that way if you want.

I want to say a word about what goes into program support.

I know most of the time people really are thinking of two major buckets of cost, one is above the line, money that goes out that door, and then the rest of the cost. But we do -- I mean, other than the money going out the door for the awards, we have really major program activities operated by PCORI. Under program support we have the Methodology Committee, all the activities associated with that. If we look at science program development and evaluation that includes all the costs associated with our advisory panels, our working groups, our taskforces, our topic briefs, all those activities, and also with PCORnet they have a lot of meetings involving all the members of the network, that's also part of this budget.

And with the engagement, dissemination, follow up contracts, some of those activities are all part of this. So, in addition to our personnel

costs, all the program activities associated with supporting our program is in this bucket.

With contracts management, a lot of it is our merit review costs is also in program support, and obviously without that, we won't have our programs. So, that's what program support is. And administrative support is, you know, Board of Governors meeting and also management in general.

So, this reflected the three major categories of cost, which is also consistent with the standard nonprofit way of classifying cost. One thing I would like to say is that every year we're refining our presentations and these details of information that the Board would need. Moving forward we will probably further refine it next year. One thing is that so far, most of our activities are really related to making the funding commitment.

We're at the point that we are growing the size of the portfolio, the number of awards we have, so we will have significant activities related to monitoring. Earlier there was a

question about what do you do when an awardee is off track in their milestones, so we will be beginning outside visits where we evaluate the performance of awards, if that is warranted. We will do programmatic site visits, maybe financial site visits, that's all part of our portfolio management plan.

In addition, there will be increased activities in dissemination, so we are also looking at, right now, all of the them are -- kind of varying program support, big buckets, so we will take another look to see, you know, as the Board members are interested in further detail, what are the things that we want to call out to give you a better sense of the program activities we have, which is now part of program support.

Here is a graphic presentation of the comparison between '14 and '15. So, in '14, our breakdown is 64 percent in program, 22 in program support, 14 in administrative, and with '15 we are trending down in both program support and admin. We hope that trend will continue.

Another thing is our staffing plan, how we plan to staff out the organization in order to support the volume of program activities we have, particularly in the area of portfolio management. So, 2014, we have a staffing plan of 164 positions. We are two weeks from the end of fiscal year, we have 160. We have 160 employees.

For example, 2015, we are requesting 53 additional positions and over half of that will be in the science department because we need more staff to manage the portfolio we have and then the rest is in engagement, contracts management, contract management we are particularly asking for positions in the post-award team that would help us support the portfolio that we have, and then administration.

I know there were some discussion earlier about our ability to recruit the number of staff we have and to on-board them, and in 2014, we have recruited and on-boarded more than 70 positions, so we are confident that we will be able to do a good job in recruiting on-boarding the new positions

that we are requesting for 2015.

And also that we are -- we do periodic employee surveys to make sure that the employees that we hire feel that they are engaged with their work and also they're enabled, and we have seen significant improvement from last year -- from last year's result. So this year we're looking at the result, looking at the areas that were done very well, but also looking at areas that we need to put more work into it to make sure that our employees have all the tools they need to do their job effectively.

Any questions? Hi, Ellen.

MS. SIGAL: Ellen Sigal. Board. So, thank you, I mean, this is a very aggressive, and I understand, probably much needed recruitment, but I guess from reading the Board material I'd have two questions. So, if you think you can recruit them, that would be good, because, again, increasingly we have less and less time until we, you know, until we may or may not be in business, so that may be an issue for recruitment.

The other issue is space. I mean, 53 employees, do we have the physical space to put them? And that's also a cost I assume you've factored into that too and other options that were discussed in the Board package, so maybe you can address that.

MS. YAN: Sure. I think our experience is that recruitment is getting easier. Last year was much harder because we were very new, our infrastructure and system were not all in place. The fact that now we have more employees, we have, you know, I think we are seen as a more established employer now than before, so that makes it easier, and also that because of -- I think the -- our funding, we are also becoming a more established funding organization, that also helps us in getting the reputation out as a good place to work because we have very dynamic programs. So, I think that has made it easier for us.

About space, yes, it's a great challenge for us. We have, I think, in a few weeks, we will be -- our science staff will be moving into a new

location just one block away on M Street, and we are also acquiring some additional space in our current building, some sublet space, so we are monitoring that very carefully, especially projecting what we may need, because it does take six to nine months to get new space online, but we're monitoring it very, very closely.

The last six months have been very challenging because we've doubled the number of staff but we have the same number of space, so we've been doubling and tripling staff in an office, people have been very patient, but the relief is in sight, just in a few weeks. We hope at the next Board meeting you get to see our new office space on M Street.

CHAIRMAN NORQUIST: I'm letting you guys go ahead. I just know it's Rick's turn. Do you want me to handle who's up or you can do it?

DR. SELBY: Sure.

CHAIRMAN NORQUIST: So, Rick is next.

DR. KUNTZ: Yeah, a very nice presentation. Two things, one is that I would

recommend that the principle way to look at overhead is to just carve out the administrative costs and do what you said, is to put together the other program costs in the research side. I think that others can ask for analyses differently, but they'd have to make a good argument. But I think you've made a very good argument that our overhead costs really are administrative costs and that we should just put them as our principle way of distinguishing them into the buckets that you talked about.

Secondly, in the out years of -- for costs, it would be very helpful to understand which of the years of the funding is being funded, so that we look at '18 and '19, are they the third year, fourth years of those grants? Are you doing any initial new grants? And I'm still not quite sure, you know, for example, are we going to fund any new grants in '18 or '19? And so that would be nice to have some kind of way to show that. Thanks.

MS. YAN: In the current funding

commitment plan, our function is that ’18, ’19 we’ll continue to fund new projects and in ’19 we’ll be funding three-year projects, that’s why as far as expenditure is concerned, you can see that, you know, we are planning out to 2022, that’s when we have to close out those projects.

DR. KUNTZ: Then why are we going down and funding them for ’17, ’18, and ’19? It seems like we should still be funding the tail end of other projects too, right?

MS. YAN: We will -- one thing about our commitment is, when we make a commitment, when we execute a contract, we make the commitment for the entire amount of the entire project, for the life of the project. So, the next time you’re seeing those projects is the expenditures that’s coming in. So, the commitment made in 2016, you know, you would see the expenditure coming in ’17, ’18, ’19.

CHAIRMAN NORQUIST: Gail, wasn’t it?

MS. HUNT: Gail Hunt. I’m a little bit concerned about just 3 percent of the budget being spent on engagement. Is that something that we can

think about expanding?

DR. SELBY: So, you know, we do engagement in a lot of ways.

So, a lot of engagement shows up as other than in that 3 percent. For example, we pay for engagement in every project that we fund. I think you will see an uptick in the combination of engagement and dissemination over time, but, you know, we worked this budget out very closely with Jean Slutsky, who is chief officer for engagement and dissemination and it was our mutual sense that this was just -- it was the right amount for this year.

CHAIRMAN NORQUIST: Allen.

DR. DOUMA: Could you talk a little bit about --

CHAIRMAN NORQUIST: You need to --

DR. DOUMA: Allen Douma. Sorry. Could you talk a little bit about beyond ’15 looking at the types of activities going more from commitment to actually monitoring and evaluating projects that are coming in? The impact on staffing, is that

going to be significant and is it going to change the numbers of people or perhaps retreading of people who are already there?

MS. YAN: Okay, yeah. I can say a few words and then maybe Joe can jump in too.

Beyond 2015, we already know that there are several -- I think that ’14 and ’15 are the two years that we are really staffing up, we have to, you know, to just meet today’s needs and probably tomorrow’s needs, but we know that there are one of two pocket areas that we still need to staff a little bit more. For example, in my discussion with Jean, talking about dissemination, because dissemination is going to be a growth area for us. We’re getting to that point. We have to do it. So, that could be some positions dedicated to supporting and managing and needing dissemination.

This year, you know, we are asking for 29 positions in the science area, so that is mainly to support the portfolio management. So, what we do is as we review our workloads and look at where we -- number one is where that new areas that need

additional support and in areas that the workload may have come down, we will look into internal remobilization of resources to make sure that we don't have idle resources sitting around.

DR. DOUMA: Do we have any quantification of what you're just talking about yet?

MS. YAN: What do you mean?

DR. DOUMA: The estimate of the pockets that need it more and how much more and the possibility where we're going to not need as many and how many that would be? Have we got any numbers that we're working with?

MS. YAN: Not really. We are beginning to discuss it, but it is pretty much on our mind to think about and to decide throughout 2015 so that in '16 when we come to you, we have a better sense.

DR. SELBY: And, Allen, it's also related to decisions we make about, for example, the number and size of awards. As the size of awards goes up, the number goes down, and it's not a 1:1 tradeoff, so you may gain -- you may need slightly less staff if you're managing a much smaller number.

We also may do more contracting for really large -- we may do contracting for the management of some of that research.

So, some of it is still in the hands of the Board to decide, so there's some uncertainty at this point.

CHAIRMAN NORQUIST: You know, I think that -- this is Gray -- it's critical is that our strategy can make a big difference [off microphone]. Right? Anybody else comment? Questions about the budget?

[No response.]

CHAIRMAN NORQUIST: We need to move --

UNIDENTIFIED BOARD MEMBER: If I could just comment very quickly. Just congratulations to Regina and her team. As she indicated or Joe indicated, you know, clearly, our level of sophistication has advanced each year and it's really because of just a ton of hard work that's gone into this.

There's a huge amount of detail -- a huge amount of detail in the materials that you have in

front of you. Obviously, Regina's only gone through kind of the highest level here. There will be other issues and other questions as the year progresses because we all understand that a budget is essentially obsolete as soon as you approve it because the real world sort of takes over. So, we will continue to come back to the Board with significant changes or significant issues that might arise over the course of the fiscal year, but I think what we have here is something that is well aligned with the story that we've been hearing throughout the year, and well aligned with the direction that I think we agree that we want the organization to go into.

It doesn't mean that there aren't many opportunities and many forks in the road coming up for us to make changes, and we have the resources to do that, and I think that's a good thing, but I think this clearly sets us on a good, clear, accountable path and that's really what a budget is for.

CHAIRMAN NORQUIST: I'm going to let

Harlan -- and like I said, but I want to also thank the FAC, Kerry, and your group, who has really done a great job kind of working on this and staying on it too. So, there's a whole team here who have really kind of worked on this. Thank you very much. Harlan.

DR. KRUMHOLZ: Thanks, Gray, and it is --

CHAIRMAN NORQUIST: Say your name.

DR. KRUMHOLZ: Harlan Krumholz, Board member, and it is, in terms of work, and you're laying it out for us so clearly, I think the Board -- I just wanted to give you what my reflection is on this -- when I see the amount of money we're spending, the amount of people that Joe needs to hire in order to execute this work, the kind of investment that the staff and others around the country are making, it reflects back to me our responsibility to ensure that there's a return on this investment for people and that for us to really focus intently on this portfolio and, you know, we started from nothing so we'll end with nothing.

And so, the question will be at the end, for all the money and effort that's expended, whose lives are better as a result, whose decisions are more informed, to what degree is the value of this information commensurate with what we as a Board have commissioned in the work that Joe and his staff are so ably pursuing, have we set the strategy adequately in order to ensure that this money is being wisely distributed and invested so that there will be benefits?

I'm just saying personally for me, it's a breathtaking presentation. It makes me feel even more pressure for us as a Board to act responsibly and ensure that we get that return, because it's clear to us that you are -- you've invested so much in ensuring that the directions that we're setting are being pursued appropriately, but for the Board, that strategy is something we have to reflect on because it's a big responsibility.

CHAIRMAN NORQUIST: Very well. Okay, other -- all right, so we need to have a motion to approve the budget and a second.

UNIDENTIFIED BOARD MEMBER: I move that we approve.

CHAIRMAN NORQUIST: Okay. Second?

UNIDENTIFIED BOARD MEMBER: Second.

CHAIRMAN NORQUIST: Okay. Now, what we're going to do is a roll call vote, but we've already done the discussion and so Bill will do the roll call.

BILL: Debra Barksdale?

CHAIRMAN NORQUIST: She's out.

MS. BARKSDALE: Approve.

CHAIRMAN NORQUIST: Oh, that's right. Debra is on the phone. I wanted to say you're not there, Debra, but you're on high for us, so thank you, Debra.

BILL: Yea or nay, Debra, on the budget?

MS. BARKSDALE: Approved.

BILL: Thank you. Kerry Barnett?

MR. BARNETT: I approve.

BILL: Larry Becker?

MR. BECKER: Approve.

BILL: Francis Collins?

DR. LAUER: Mike Lauer sitting in, approve.

BILL: Allen Douma.

DR. DOUMA: Approve.

BILL: Arnie Epstein? Alicia Fernandez?

DR. FERNANDEZ: Approve.

BILL: Christine Goertz?

MS. GOERTZ: Approve.

BILL: Leah Hole-Marshall?

MS. HOLE-MARSHALL: Approve.

BILL: Gail Hunt.

MS. HUNT: Approve.

BILL: Jesse is not with us. Richard Kronick?

MR. KRONICK: Approve.

BILL: Harlan Krumholz?

DR. KRUMHOLZ: Approve.

BILL: Richard Kuntz?

DR. KUNTZ: Approve.

BILL: Sharon Levine?

DR. LEVINE: Approve.

BILL: Freda Lewis-Hall?

DR. LEWIS-HALL: Approve.

BILL: Steve Lipstein?

VICE CHAIRMAN LIPSTEIN: Approve.

BILL: Gray Norquist?

CHAIRMAN NORQUIST: Yes.

BILL: Ellen Sigal?

MS. SIGAL: Yes.

BILL: Harlan Weisman?

DR. WEISMAN: Approved.

BILL: And Bob Zwolak?

DR. ZWOLAK: Approve.

BILL: Motion passes.

CHAIRMAN NORQUIST: Thank you. Okay, you guys can move and the next topic we're going to consider the posting for public comment of PCORI's draft proposal for peer review and release of research findings, and let me be clear here, we're not approving this policy; all we're asking to do is to be able to post it for comment.

So, Joe, this is yours. Joe?

DR. SELBY: Thank you, Gray. So, this is a draft policy that has been developed by PCORI

staff and vetted with each strategy committee and presented to you once before two weeks ago on an open Board call.

And we're bringing it back because there were some considerations raised by Francis and reflecting NIH concerns about some of the language and we've worked closely with staff from NIH in the intervening two weeks and I'll report quickly to you on where we've gotten and I think I can say that we -- that both NIH and PCORI are satisfied with this as a document to go out for public comment.

So, the key points are that we're told to do two things in the legislation, one, is to conduct a review of our primary research, and that's for two purposes, one is to make sure that it's rigorous, that it's valid research, and the second is to make sure that it adheres to the methodology standards. So, this is something that we have to do with our primary research, and it's also, as we've pointed out, something that the standard peer review processes of journals don't do

in a timely way and they don't -- we can't count on them to formally assess whether the studies adhere to PCORI's methodology standards.

The second thing we're trying to do and told to do is to release the research findings as early as possible and no later than 90 days after the conduct or receipt of the research findings.

We have to make it available to clinicians, patients, and the general public in a comprehensible fashion. We have to include considerations about the effectiveness in certain subpopulations, that's taking a look at treatment heterogeneity. We have to -- heterogeneity based on risk factors, based on comorbidities, based on genetics. And we also have to describe the process and methods used, including conflicts of interest and limitations of the research.

So, this has to be in a report that we release to the public and put on the PCORI website and other places within 90 days after the receipt of the report.

So, how do you reconcile this? One

principle I want to emphasize is that except in circumstances where other regulations dictate a different course, we've elected to peer review the findings before we release them to the public. We don't think it makes sense to release findings before they've been peer reviewed and then have to go back and change them. We think that's a disservice to patients and clinicians and confusing, to say the least.

So, our principle in the whole design of this is based on peer review first and then post.

We've already said that we've discussed this before.

So, the proposal, essentially, has the following elements that every project be registered in a public registry, whether that's clinicaltrials.gov for empirical research, whether it's randomized trials or observational studies, the registry of patient registries, should we fund a patient registry, the development of a patient registry, and PROSPERO, which is a registry for systematic reviews.

We require a draft final report. The awarding institution is responsible for submitting to PCORI a draft final report for peer review three months after completion of data analysis, and this is a tricky -- this has been one of the trickiest parts is to set this date, but this is our considered opinion after consultation with NIH and after looking at the FDAAA legislation and after considering the kind of research we're conducting.

So, three months after the completion of data analysis, this is the date that will have to be specified in the contract and set in milestones.

The PCORI peer review, PCORI will, in fact, manage a peer review process for the final report using a combination of staff and probably substantially contracted resources rather than substantial staff resources. This is what the final report has to contain: it has to contain the main study results, it has to list the methods, discuss the issues related to differences by subpopulations, discuss by subpopulations risk factor levels and comorbidities, it has to discuss

the limitations of the findings, the needs for further research, it has to have tables, and it definitely has to have conclusions.

It must have a 500-word abstract intended for medical professionals and scientists. It must have a results table, and this is relevant to those that are posted on clinicaltrial.gov, it must have a results table to follow the clinicaltrials.gov format and requirements and must be submitted to clinicaltrials.gov and also submitted to PCORI. And it must have the ancillary information mentioned in the legislation about the entity, the investigators, and any disclosures or conflicts of interest.

So, making the public research -- the research findings public includes both providing information for various audiences, that is a lay abstract -- an abstract of the results intended for lay audiences, and a public posting on PCORI.org and submission to clinicaltrials.gov. So, PCORI will post information for patients and consumers on our website and the clinicaltrials.gov will host

the abstract -- we will also have the technical abstract and table on our website, but clinicaltrials.gov will host the technical abstract and the results table that they require.

So, this just highlights three major concerns that NIH raised at our last -- on our call. First that the FDA -- I'm not sure what authorizing and I'm not sure what the other A stands for, the other two As stand for, Ellen, you may know or Freda, you may know, but at any rate, it speaks to requirements for certain types of trials to publish their results in a more timely way on clinical trials. We clarified that we certainly in no way intend to interfere with that or slow it down and also that the awardee institution, not PCORI and not the investigator, are responsible for ensuring that this information is submitted to clinicaltrials.gov in a timely way.

The second is a clarification of the date, which triggers the beginning of the process, I already alluded to that. We changed the trigger date from the end of the contract to three months

after completion of data analysis because a lot of our work is, in fact, data analyses and that's a date that can be specified beforehand. And the third is that we need to provide in the document some more detail about PROSPERO and the Registry of Patient Registries to make it more parallel with what we said about clinicaltrials.gov.

The facts are that most -- the vast majority of our research will be relevant to clinicaltrials.gov, not these other two. We want to collect input about these two from the public comment period and during that period, we will also collect more information on these two and add it to the final document.

So, those are the three concerns that we responded to in consultation with NIH.

So, the timeline is that we hope that the Board will vote to approve the posting of this draft proposal for public comment today. We will then launch the public comment period. We're all prepared with a website to collect comments and to show those comments to the public as they come in.

The comment period will last 54 days from today through November 7th. We will analyze and synthesize this data in collaboration with a contractor, who has already been identified and is engaged, and we will provide a report -- we will receive a report from the contractor on January 10th of the analyses of the public comment. The strategy committee review period, all three strategy committees will review this between January 16th through February 17th and we will submit it to the Board for a vote on the revised proposal at the end of February.

So, that's it. Any questions?

CHAIRMAN NORQUIST: So, let me do it this way. Let's kind of make the order go right. We have a motion to approve this for posting, and then we'll have the discussion. So, if I could get a motion to move this, thanks, Bob, and then a second? Okay. All right, now we'll open it for discussion. Harlan Krumholz.

DR. KRUMHOLZ: Thanks, Gray, and I know we did discuss this on an open call, but the more I

thought about it -- this is Harlan Krumholz -- the more that I am unsure what it is that we're requesting in the sense that we have some legislative mandate in order to do the peer review, but here are my concerns. I don't want to delay the posting. I think the rapid cycle time for dissemination of results is important. I think that there's a lot of fuzziness around when do you finish the analyses?

I mean, I know papers that never finish the analyses, so it sort of can go on for a long time, so how that gets negotiated and overseen is important. And what role PCORI is playing in the peer review is not exactly clear to me. What if people aren't responsive? How deep are we supposed to go? What if we disagree around the edges?

I mean -- and I know this is imposed on us by the legislation so these are all questions for us to respond to, and one way to do that is to go out for public comment and see what people say, so I'm not exactly sure where we stand, but I just wanted to express that this issue about peer

review, which is not done elsewhere, is being asked to be done here, to me seems very tricky.

And, you know, another way to do it is for people to post their results and for us to post our peer review, and actually allow the community of people to see that peer review as opposed to the notion that there is one right answer, here's the investigator's thought about the answer, by the way, we provide our critique of the answers, other people are able to put in their thoughts about it, it eventually gets published, it creates a dialogue around the results.

I mean, I'm just thinking out loud. I think it puts us in a tough position if we're approving that research, I mean, if they're handing it into us and are we a peer reviewer that basically says, yeah, you're ready to be posted or published. In my experience, this never ends, especially if we're in an open science era, more people get to look at the data, more people have different opinions about the data, science progresses. I always quote Francis, science is

progressive and self-correcting, and I ended by saying, if the data are available for people to work with and if there's freedom for people to exchange different views.

And this is just meant to share not so much a response to it but just to share with the Board some of the thoughts I had as I really started thinking about this peer review process.

CHAIRMAN NORQUIST: Go ahead.

DR. SELBY: I feel your pain, in a way, and we've been working on this for a long time because of this curious, almost conflict in the legislation. Two things, though, we're very aware that we're instructed to publish a substantial amount that's intended for the lay public, for patients, and that was one of the thoughts that made us feel that we actually do want to peer review before we post it because it's going to be on our website for patients to look at.

The second thing is, we didn't think we could get around this notion of peer reviewing to ensure adherence to the methodology standards by

saying that, don't worry, a journal will take care of that. So, those were the two things that drove us to feel that we needed our own peer review process.

DR. KRUMHOLZ: But one of my questions -- won't people say that if at the end they're not adhering then we haven't done our job either in the approval of the grants or in our monitoring of it through the process? And secondly, what if I, as the investigator, disagree with your peer review? What are you going to do then in the sense -- and, you know, you and I have a lot of experience as investigators don't always agree with the peer review that comes our way and so how is that -- it's just another issue to manage?

DR. SELBY: I think that is a very good question, a question of what if at the end of the day we do disagree about the peer review and I don't know that I actually have an answer for it at this point. I think ultimately one of the reasons that NIH pointed us to this notion about the end of contract was if we said three months after the end

of the contract, there was really no other lever to use with people to get the research done. Now, we built it into milestones and the final payment doesn't occur until we've got the final draft peer reviewed.

So, that gives us --

DR. KRUMHOLZ: [Off microphone.]

DR. SELBY: You like that? Okay, good. But I think that's a good public comment, Harlan, that we're going to need to figure out what is our stance on research, which after everybody's best efforts there's a disagreement? It's not like we can -- if you're peer reviewing for a journal you just say, send it elsewhere. We don't want to say that. We want to say, ultimately, post it. So, I think we're going to have to work on that a bit.

CHAIRMAN NORQUIST: Allen?

DR. DOUMA: Several things, one is more profound and a couple are more detailed, I guess. I think that we need to have more respect for patients and caregivers that they are able to look at something and if it says, this is in peer

review, it's not final, it's okay. We -- too often as professionals we don't respect patients and people enough and I think we ought to recognize that as a flaw in our own behavior.

I think -- a couple of questions, one is in the initial discussion, I didn't see it here in this presentation, it wasn't clear how long the iterative process between submission of the results, our peer review, sending it back, the PI reviewing it, agreeing or disagreeing with what was said, it comes back to us, it goes back and forth, and how long can that possibly take? If we allow four or five iterations and they're a couple of months each, it's going to take a long time.

So, it would be good to have some kind of timeframe for that.

And another question I had back when this was first presented and maybe you have an answer now, is it, are we going to be able to apply this to existing contracts or is this a process that only can be -- particularly if there's any punitive aspect to it -- of contracts are going forward in

the future?

DR. SELBY: You know, I think that the first contracts affected are those from the pilot studies, which are coming in now, the results are coming in. I think we will try to apply it as best we can but knowing that, you know, some of the -- like, for example, this date of the -- final data -- the end of data analysis has not been set for the pilots.

I think we will try to, without doing a bait and switch with the awardees, we will try to apply as much of it as we can to all of the funded research.

Again, I think, you know, PCORI also has an interest in at least having a rough look at what we're going to post on our website and making certain, through a peer review process, that we're comfortable putting it on our website. I just -- so, it's not out of a lack of adequate respect for any stakeholder group that we do that, it's out of our own due diligence.

DR. DOUMA: Well, consider what Harlan K.

said is, if it's a totally transparent process, then we can do all of that. We can do both. And I think, again, that people can see that this is in process and not act hastily based on information that's in the --

DR. SELBY: And, in fact, that may be the solution to Harlan's question is that if in the end we don't agree, we post their findings and any concerns we have about them.

CHAIRMAN NORQUIST: Harlan Weisman.

DR. WEISMAN: Yeah, Harlan Weisman, and I have, I guess, supplementary comments to Harlan Krumholz and what Allen Douma was saying, and it's really around our mission, which reads, "PCORI helps people make informed healthcare decisions and improves healthcare delivery and outcomes by producing and promoting high integrity, evidence-based information that comes from research guided by patients, caregivers, and the broader healthcare community."

And, you know, you insert that into our vision, which is that we make sure patients and the

public have the information they need to make decisions that reflect the desired healthcare outcomes, it means that somehow we're not only just posting an abstract or here are the results and they've gone through peer review, but there ought to be some kind of interpretation and guidance function about what the data -- not necessarily that we have to interpret the data for clinicians and patients, but at least guide them on how they might be able to interpret it and use it and incorporate it into their decision making, which is far more complicated, I think, than what's being outlined here, but it seems to me to be the most important end product.

I think it's wonderful that it will go through scientific peer review and be published in high quality medical journals. I don't think that's PCORI's major mission.

PCORI's major mission is to provide patients and clinicians and others, their families, with the information they need to make high quality decisions. That's not going to come out of the New

England Journal of Medicine, with all due respect to that journal, or the Lancet or any other one. It's going to come from something, hopefully, that PCORI does.

One last point is, as Harlan K. was talking, I was thinking maybe of a wiki-like process of posting something, providing our interpretation on its utility, and perhaps the investigators' viewpoint on that, and then allowing, as wiki does, a process of discussion, debate, and perhaps resolution.

CHAIRMAN NORQUIST: Okay. Larry, and then Rick, and then Bob.

MR. BECKER: This is Larry Becker. Just a question. In the final report, did we consider a paragraph on what this means to patients?

DR. SELBY: Well, you know, the legislation is really explicit. They say, we want conclusions, their applicability, their limitations, their possible differences in different patient subgroups. So, I think the legislation makes it very clear that this has to be

a useful document to patients and I think that is part, again, of where the peer review process would come in. It's not -- you know, 300 awardees are not uniformly going to crank out these abstracts with the limitations and the conclusions and interpretations in the subgroups without a little guidance, and that, I think, is what our peer review would focus on that in the methodologies.

DR. WEISMAN: You know, there's another set of peers that have to be included that aren't typically included in journal review, and those are the patients and clinicians and family and caregivers, the other stakeholders, who should be part of the process, and have we thought about that?

CHAIRMAN NORQUIST: Okay. Rick?

MR. KRONICK: I think all of this conversation suggests that to get really useful public comment, providing some more information and perhaps some questions about what this peer review process might look like would be helpful. So, if we're clearly considering a number of options of

what the peer review process might be, you talked about, you know, having a contactor but, you know, what might get done, and if we want more useful comments providing a little more on either what we're thinking or what the options are in what we're thinking, we're likely to solicit far more useful comments.

CHAIRMAN NORQUIST: And that was Rick Kronick.

MR. KRONICK: Rick Kronick, Board member.

DR. SELBY: So, Rick Kronick, in fact, the contractor has been given a set of questions to -- explicit questions about the reports to try to solicit some -- in areas that we have doubts about.

CHAIRMAN NORQUIST: Bob?

DR. ZWOLAK: Bob Zwolak, Board member. I think this is going to launch us into a new area, certainly investigators are used to peer review, but now they get the distinct honor of undergoing dual peer review and it may be different, it may be different metrics proposed or provided in the PCORI peer review, but I think we may need to think of

this a little bit in terms of an iterative process because we may come to the point where, in some cases, where the scientific journal peer reviewers may have different thoughts about the data or the presentation than the PCORI peer reviewers. And so there may, in fact, be some controversy we have to look at or settle over time.

DR. SELBY: Yeah, I wanted to clarify that. In the past, researchers have been very troubled if an agency wanted to peer review their work before they could submit it for publication, and we're not saying this. Our policy does not go there at all. They can have submitted a paper before they submit their final report to us.

They can submit a paper while they're undergoing our peer review or we'd actually be happy to invite them to share our peer reviews with a journal they're applying to later, so we are not building in this delay or interference with investigators' ability to publish.

CHAIRMAN NORQUIST: I think that's all. Okay, so we'll call the question. All we're voting

on, and this can be by voice vote, is the ability to put this up for comment. We're not approving this policy. Obviously, we'll take all the comments in consideration. Yes, Harlan?

DR. KRUMHOLZ: I just wonder about the response to Rick's point in the sense that we're voting broadly for this public comment but we're not saying how it's going to be proposed for public comment and I just didn't know if just, Joe, you wanted to -- how you wanted -- just to respond -- the point, I think, is that the more specificity and clarity around this, the better quality of the public comment, and I think raising some of these particular questions and issues, these are the pressure points. In other words, here's the policy, here's some of the pressure points around this policy that if people have views, we would welcome.

And I think you could cluster them. I mean, for investigators, how's it going to feel to get two peer reviews? How are you thinking you're going to manage disparate reviews? I don't know.

But there are -- it would be great to -- I'm not just trying to get criticism of our policy, but more like crowd source ideas and to make sure that we're conveying an openness.

We're trying to balance our legislative mandate, our urge to get this stuff out, our urge to get it out in a way that's reliable and trustworthy, recognizing that there could be additional burden imposed. We're looking for good ideas. Here's our first shot at this, but we're eager to hear suggestions about ways that we could do better.

I think that kind of tone could be very inclusive and supportive rather than, you know, here it is, and throw something in if you want, but we're already, you know, locked in on this.

CHAIRMAN NORQUIST: I think Rick wants to add --

MR. KRONICK: Rick Kronick, Board member, and I guess Allen Douma's earlier comment, you know, it would be useful in getting public comment to provide the public some sense of what we're

expecting in terms of timing, you know, that kind of information.

CHAIRMAN NORQUIST: I think that's very critical. Joe, do you want to respond?

DR. SELBY: Well, you know, I like where this is going and I'd like to say that I think that we can continue to take your suggestions, either now or even after this meeting, on specific questions. One of the specific questions that we asked was, in fact, what is your response to the proposition that these reports should be peer reviewed? So, we invited the public to actually weigh in on this notion that we were doing our own peer review. That's one of them.

Then I think I looked at it briefly with Bill last week and I think there's about five to six sections where they're invited to comment on different portions of it, but more specific comments would be -- I totally agree, it would be very indicative of our sincere interest on tough questions that we've wrestled with.

DR. DOUMA: Gray, quickly, on the process.

When we put this up for public comment, and just an example, I respond with a question, will other people be able to see my question?

DR. SELBY: Your comments? Yes.

CHAIRMAN NORQUIST: So, are we ready? Are there any other comments about this? Do you have a game plan here? Do you feel comfortable with --

DR. SELBY: The only -- yes. I feel very comfortable. The only thing is I'd love to, you know, invite Harlan and anybody else to submit specific questions that you think make sense to put on this solicitation of public comments.

CHAIRMAN NORQUIST: That's kind of a friendly amendment, I think, that is helpful. But let's get a deadline on getting those questions in so we don't have this -- so, if we could -- if we're going to post this, we're going to post it soon.

DR. SELBY: Let me just ask Bill what his take on -- is on whether -- we actually want to get this posted today just so that there's -- you know, the legislation says 45 to 60 days, we'd like to be

as close to 60 as we can. But if we post it today, Bill, we should be able to -- we should be able to make some modifications. Few people are going to respond in the first one to two days.

BILL: We have a couple of possibilities; one would be to walk through what we have now. We have a couple of folks to make sure that some of those issues are covered. I think many of them are but there's always opportunities to get more specific, and I certainly agree with you, Harlan and Rick, that the more guidance we can provide, and I think we've provided more than one might expect in a general public comment period on something like this, can be helpful.

So, we can certainly hold off for a day or so to get that done if you wanted to make it official.

DR. KRUMHOLZ: One question I had, Bill, about this public comment, will the public comment be visible -- I mean, sometimes people are just throwing -- like, if I make a comment, will other people see my comment?

BILL: Yes, all comments -- all comments will be displayed in real time.

DR. KRUMHOLZ: Yeah, so for me, as a living document, I think it's okay. I mean, you can go out and encourage people to even post questions and then there may be some conversations that accrue. I think that should be the spirit of what we do rather than say here's the perfect piece.

I mean, but I think there's a part about encouraging people and getting them off the mark, so if you can start with a few key questions for different constituencies, I think that's great, but I don't think we have to feel that we're being exhaustive because it starts a conversation, I think that would be great, and I think you're also, from the investigator point of view, engaging Academy of Health and some of the others to get people engaged in talking, the patient engagement groups, our PPRNs, you know, others to kind of get engaged and help us would be really terrific.

BILL: And that's part of the outreach

plan, the combination of live -- a live event webinar and additional outreach to solicit comments.

I think the only open question is, are there any critical guidance type questions that should go in from the get go or are we ready to go now in the spirit that you suggested, Harlan?

DR. WEISMAN: Would you allow -- I mean, I don't know what Joe had in mind, but in terms of Board members providing additional comments or questions before you post, what do you think about the idea of having Board members be part of the public and offer comments and questions that others, including PCORI staff can respond?

BILL: Yeah, it's completely fine. I mean, one of the models we used here was -- remember, we've done this twice before, we did it with priorities and agenda and we did it with the methodology report and standards, and in both cases we saw the Board and others who have an official PCORI relationship as among those whose comments we would value.

CHAIRMAN NORQUIST: Joe, I think we have a plan then and it sounds like we can make it as transparent as possible and we may not have all the right questions, but let's see what the field says, right. Okay.

So, I'm going to call the question.

All those in favor of posting these?

[Chorus of ayes.]

CHAIRMAN NORQUIST: Anybody opposed?

[No response.]

CHAIRMAN NORQUIST: Any abstain?

[No response.]

CHAIRMAN NORQUIST: Okay, it passes. Thank you. Steve, you're on for the Governance Report.

VICE CHAIRMAN LIPSTEIN: I am the last agenda item between you and your lunch, so I will try and be brief.

Members of the Board will remember that about 15 months ago we commissioned an evaluation of our governance processes and structure that we had put in place during our first three years of

PCORI's existence and out of that evaluation process conducted by Marla Bobowick and Debbie Hechinger there were a series of recommendations made that you all approved.

Actually, you approved at this meeting about a year ago and just to remind you that as a consequence of approving those recommendations, we approved the Strategic Plan at the beginning of 2014. We reconstituted the strategy committees of our Board and recharted them in the first quarter of 2014, which included the Scientific Oversight Committee, the Committee on Engagement, Dissemination and Implementation, and the Research Transformation Committee.

And then a lot of that activity just resulted in the budget that you approved earlier this morning.

The one thing that we did not approve a year ago, the one question that we didn't -- or recommendation we did not approve a year ago that we delegated to the Governance Committee of our Board is the question of whether or not we should

have an Executive Committee of our Board.

So, I am reporting to you today in a capacity as the Chair of the Governance Committee of the Board and other members of that committee are Robin Newhouse, Gray Norquist, Allen Douma, and Sharon Levine. So, I am reporting our recommendation as a committee to you.

The three topics that we have discussed at our meetings between the last Board meeting and today have to do with this Executive Committee, guidance on voting, and the status of selecting topics for Board development education, and I will speak quickly on all of these but spend a little bit more time on the Executive Committee.

One of the reasons for having an Executive Committee, as we've discussed, and when we reviewed our recommendations a year ago, is to basically either to allow staff to get needed Board approvals between meetings or to provide final approval of matters that have already come before the Board, or to approve items that just don't warrant the time and attention on this agenda of the full Board.

When we evaluated those possibilities for chartering an Executive Committee in the context of both our authorizing law and how that law limits what we can delegate to an Executive Committee and what we must do in public and open session, and then as we evaluated that in the context of D.C. not-for-profit law as to also what needs to be done at the full Board level and not at the Executive Committee level, we found that there were really no significant matters that had arisen during the first three years of PCORI's existence that would have been delegated to an Executive Committee, and we tried to write a charter for one and we found that there was just nothing to put in the charter.

So, what we did was, we realized that there were other avenues in terms of what the executive director and staff had available to them in terms of the executive director meeting with or discussing issues with Board leadership and with the committee leadership, and the Board has authorized already the chairperson and vice-chairman of the Board to approve certain

expenditures as part of the budget that you just approved earlier today.

So, after deliberating on this for two meetings and trying to come up with a charter, we just are recommending to the full Board that we not authorize an Executive Committee at this time, that there just wasn't sufficient responsibility that could be chartered to an Executive Committee that doesn't need to be done in front of the full Board, and I'm going to talk a little bit about that in just a minute.

I'll come back to that recommendation, but I'm just going to move through this so that I can provide the full context.

As you will see, when it comes to voting at Board meetings, whether we're voting at open meetings of this Board in face-to-face meetings, or voting over the phone in open meetings, we really looked into whether or not we need to do voice votes, which are less time consuming, or roll call votes, which are more time consuming, and so what we decided is that voice votes are appropriate for

approving the minutes and things that are on the consent agenda for each Board meeting or each committee meeting, and that any member of the Board or any member of a committee can ask that something be removed from a consent agenda so that it can be discussed individually and voted on individually.

But these are the things that require a roll call vote and these are also the things that this Board must do as a full Board, and that we would never delegate to an Executive Committee. We approve all awards or slates of awards, we approve the budget, we approve any matters that require, really, a heightened level of approval, such as our bylaws, and then again, by statute, the full Board must approve of everything related to our National Priorities, our Research Project Agenda, the Methodology Standards, and the Peer Review Process.

So, again, there's just a very -- and because a lot of our work has to be done in open forum, there just isn't an opportunity to delegate much to an Executive Committee.

So, the last thing I will talk about is

what we -- the third item on our Governance Committee agendas, these are the topics that we have already covered with you as a Board. You wanted much more insight into PCORI's research portfolio and the impact of that portfolio, and these are the topics that we have covered already with you as a Board.

You're going to hear more about improving healthcare systems this afternoon, and Dr. Kronick is our featured speaker this afternoon on something you also wanted to know more about, which is the expenditures out of AHRQ's PCOR Trust Fund Portfolio.

MR. KRONICK: Just a slight correction, I'm not an MD, which it says on the last slide, so just in case anybody's looking.

VICE CHAIRMAN LIPSTEIN: Neither am I, but I never admit it. That's true.

And then these are the other topics that you all have submitted to our Committee and we will prioritize these and put them before staff. But again, a broader perspective into the healthcare

research world and how we fit into it, how what we do differs from other organizations that are interested in healthcare research, an overview of healthcare infrastructure funding in research, so the PCORnet that we are establishing, a look outside of the United States to see how other countries approach PCORI's mission, and then again, an effective review of our Board of Governance tools and resources. One of the suggestions Dr. Krumholz came up with was, you know, when is the right time to do another evaluation of our Board and this new structure that we put into place.

As you know, that's a time consuming activity and not inexpensive, but it's a very thoughtful suggestion and one we want to put into the schedule and sequence for our next governance committee discussion.

And so, these are the committee chairs, and so, Gray, the only thing I think we need is an approval of our recommendation not to charter an Executive Committee, and while I'm not sure we ever vote on recommendations like that, we just wanted

to make sure we close the loop and let the full Board know that we evaluated this recommendation thoroughly and we just don't believe it's necessary that we charter an Executive Committee at this time.

I would invite any of my fellow committee members -- Robin, Gray --

CHAIRMAN NORQUIST: -- when I'm on the Hill where the attorney is talking to me --

[Laughter.]

CHAIRMAN NORQUIST: Very good solution that she has, so the easiest thing to do is just have the Board approve our recommendations. We don't necessarily have to vote on the fact that we have or don't have anything.

VICE CHAIRMAN LIPSTEIN: Okay. I'll make a motion to that effect, that you approve the recommendations of the Governance Committee.

CHAIRMAN NORQUIST: So, thank you.

DR. DOUMA: Just as a member of the Committee, I second everything that Steve is saying and it's remarkable how fast he can talk.

[Laughter.]

CHAIRMAN NORQUIST: CEOs, they can talk fast.

MR. BARNETT: This is Kerry Burnett. I don't want to slow things down.

Steve, you have your slides on the voting, you said voice votes will generally be used to take action on minutes and consent agenda items and then you have roll call votes are required -- there are actually a lot of other matters that are in between there and I'm assuming that you're not intending to suggest that voice votes will only be used on those items, that we'll continue to use voice votes on everything else.

VICE CHAIRMAN LIPSTEIN: That's correct, Kerry. I think the word "guidance" here is important.

We were just providing guidance on the things that the Committee had discussed and if there are other kinds of votes that require guidance, they should be directed to the Executive Committee or directed to our general counsel.

CHAIRMAN NORQUIST: Yeah, trust me; we will definitely do roll call votes when I'm told we have to do roll call votes.

UNIDENTIFIED BOARD MEMBER: We'll only use roll call votes when we have to.

CHAIRMAN NORQUIST: When we have to, yes. We were trying to get the voice votes -- right, exactly.

Okay, any other discussion?

[No response.]

CHAIRMAN NORQUIST: Okay. All those in favor?

[Chorus of ayes.]

CHAIRMAN NORQUIST: Anybody opposed?

[No response.]

CHAIRMAN NORQUIST: Any abstain?

[No response.]

CHAIRMAN NORQUIST: Okay, anything else? Is that it?

[No response.]

CHAIRMAN NORQUIST: We are adjourning for lunch and we should tell the people on the -- we're

coming back at 1:15 Eastern daylight time.

So, for those of you who are listening in, we will be back at 1:15 eastern daylight time.

[Whereupon, at 11:33 p.m., a luncheon recess was taken.]

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

[1:25 p.m.]

CHAIRMAN NORQUIST: Thanks. So, we're back. The Board is back in in-person session and at this point we will start the panel discussion with the PCORnet Clinical Data Network and the -- oh, everybody's ready. That's wonderful.

Okay, so we'll let you all introduce yourselves and I think --

DR. SELBY: -- and then hand it over to Rachael.

So, it always delights me to remind the Board that PCORnet was their idea, definitely not mine in the beginning, and it's one of the areas where, thank goodness for the Board, having been involved in this kind of research before I was a little on the reluctant side, but this PCORnet has turned into a world of its own and part of our mission today is to kind of bring PCORnet and PCORI, and the PCORI Board, particularly, closer together.

PCORnet is all consuming; it involves

1,000 researchers and their affiliated patients and clinicians. One hour in normal time is accomplished in 15 minutes, so there's a 4:1 ratio, we accomplish four times as much in PCORnet and time flies by four times as fast in PCORnet.

I think we underestimated the potential that PCORnet has to transform the way clinical research is done. We underestimated the interest of the country in seeing clinical research transformed, whether that be at NIH, whether that be in academic medical centers, whether that be in patient organizations, whether that be in PhRMA.

So, much more exciting, much more challenging, much more engrossing than probably any of the four PIs who are visiting us today thought or any of the others, and I'm going to now hand it over to Rachael Fleurence, without whom PCORnet wouldn't be -- I mean, Rachael really is the program director for the entire methods portfolio, but in her spare time she leads PCORnet and she'll tell you too that it's pretty exciting and engrossing.

So, I thank Rachael and the four PIs who Rachael is going to introduce coming here today, and I really -- we want to hear presentations and then have you engage with our visiting PIs to get a better sense of what's going on and where PCORnet is headed or could be headed.

MS. FLEURENCE: Thanks, Joe. So, I'm delighted to introduce four of our PPRN and PPRN PIs that have graciously agreed to come here today and make some presentations to you. So, I'm going to introduce them. At my right, Tom Carton, who's the director of analytics at the Louisiana Public Health Institute. He's the PI of our Louisiana CDRN.

Next to Tom is Rachel Hess, who is an associate professor of medicine at Pittsburgh and a professor of medicine at the University of Utah School for Health Sciences, and she is the PI of our Pittsburgh CDRN.

Holly Peay is the PI of the Duchenne Connect Patient Report Registry. She's also the director of the Duchenne Connect Registry and she's

the vice president of education and outreach for the parent project Muscular Dystrophy Project.

And then finally Sharon Terry, who is the PI of the Community Engage For All Network or CENA. She's the president and CEO of Genetic Alliance, which is a network of more than 10,000 organizations.

Sharon's list of commitments to PCORnet is endless, so I won't read them out, but she co-chairs our Governance Taskforce and is also helping us do a lot of groundbreaking work in privacy.

So, with that, I'm going to turn it over to Tom for the presentations.

MR. CARTON: Thank you very much, Rachael, for the introduction and thanks to everyone for giving us the opportunity to speak to you today. My name is Tom Carton and I'm the PI for the Louisiana CDRN and I work for the Louisiana Public Health Institute.

The overview of our network is to facilitate efficient and conductive patient-centered comparative effectiveness research by

establishing a data network with the clinical data records of more than one million patients across the state of Louisiana.

We focus primarily on three patient cohorts, each of the CDRNs was asked to choose a high prevalence cohort and a low prevalence cohort and we were all given the obesity and weight management cohort. So, our network chose diabetes, leveraging a number of years of work in diabetes care management that was really initiated under the Beacon program that formed the Greater New Orleans Health Information Exchange in 2010, and sickle cell disease, also taking advantage of some collaborations with both Ochsner Health Systems and Tulane University that have done sickle cell research over the years.

Our network partners are Louisiana Public Health Institute, the next acronym, PATH, is not Rachael's PATH, but Partnership for Access to Total Health, which is the evolution of the Greater New Orleans Health Information Exchange, which was that Beacon project.

Pennington Biomedical Research Center and LSU Health are both affiliated with the Louisiana State University System. They used to run the public hospital system, which has since been privatized, but still has use agreements with each of those contributors and their harmonic data warehouse.

Tulane University Medical School and School of Public Health. Tulane University is also a contributing member of PATH, the Greater New Orleans Health Information Exchange, and Ochsner Health Systems, which is one of the largest health systems in the state of Louisiana.

This slide is designed to just describe the cohesion across our partners. We view the project as a team of teams with research, engagement and data. That's the way we're organized. I know each network is organized differently. From a research point of view we have obtained agreements to work through IRBshare for Institutional Review Board coordination, including protocols and consents and so forth.

We've also established cohort-specific advisory groups that identify patient centered research priorities. These are made up of clinicians, researchers, and patients, from each of the contributing health systems and are built around our three cohorts: diabetes, sickle cell, and obesity.

From an engagement point of view, we are building a pragmatic trial application suite on tablet computers, integrated alongside of EHRs, but not directly through each individual EHR. We have about 10 or 15 different EHR systems across the network, some of our networks are networks of networks.

The Greater New Orleans Health Information Exchange has 12 FQHCs in the greater New Orleans area, so we really needed an EMR-Agnostic system that could communicate with EHRs, but at the same time not be beholden to each and every -- and that application suite is in three pieces allowing us to collect patient-reported outcomes, to allow for electronic recruitment to clinical trials and

clinical trials management application. The one that's in development right now is getting patient-reported outcomes and the recruitment and the management app are coming in subsequent phases in Phase I, but just not within the first six months.

We've also established a Health in Our Hands Patient Network, which is the patient-facing side of the LACDRN that patients can send into. They are then engaged bi-directionally, we send information to them, dissemination of findings, potential clinical trials, news, and also engage them to share patient reported outcomes and patient generated data.

From a data point of view, our partners have contributed to the common data model. We have 2.3 million patients in our data center at this point. We've established a global patient identification system for de-duplication of patients because our systems are complicated. One of the formal public hospitals was bought by Ochsner, so their data is in the LSU system and it's in the Ochsner system. We also have patients

that move in and out of systems throughout the greater New Orleans area, so we've worked with CAPriCORN, Chicago's CDRN, to establish a global patient ID that uses has algorithms and doesn't require sharing of PHI.

We also have an informatics solution that's capable of linking the clinical and the patient-generated data. In the early stages of collecting patient-recorded outcomes, it's not really that difficult, but further down the road when we would like to be able to engage patients outside of clinical settings and collect patient-reported data from centers and so forth, we needed an open stack solution to be able to do that.

This diagram overviews the patient and provider representation and decision-making. The former patient involvement and governance comes in the patient board and the clinician board. The co-PIs are the ultimate decision makers of the body, which I'll explain in one of the challenge pieces.

The lower part of the diagram are informal methods of capturing patient and provider

perspectives to inform network activities. A couple of concrete examples of this is the patients have vetted our tablet patient-reported outcome app suite and have also vetted a roughly one-minute video that describes Health in Our Hands to patients as they are seen in health systems.

Of the successes, first is the rapid design and procurement of this integrative informatic solution, which involves not just the clinical data warehouse and the global patient ID, but management of patient-reported outcomes, patient-generated data.

Partnership co-development of the LACDRN app suite and patient engagement strategy, that is EMR-agnostic and it was difficult for some of our systems to swallow at first, but after subsequent discussions and understanding of the necessity from the network's point of view, we were able to get that buy in; and then also engagement of patients and clinicians in network governance, decision-making and priority setting.

Some of the challenges, network partner

decision-making, so the co-principle investigators, not in every case, were the final decision makers for that organization, so the co-PI from the Tulane University Medical School has to constantly walk HCA through the process and get HCA's buy-in in both the clinical, the informatic side, but also the patient-engagement side. The same is true with our co-PI from Pennington Biomedical, who works very closely with the LSU Healthcare Services Division, which holds data that is now in the hands of a number of private partners, owned by a number of private partners.

So, ultimately, after a number of iterations, we decided that it's the co-PI's responsibility to bring their organizations on board. Now, each of these organizations sit on our administrative board, so it's not like they don't have wider integration with the rest of the team, but really we needed somebody to be ultimately responsible for that decision making. And then, achieving a balance between network standardization and nodal customization.

I think patient-reported outcomes is probably the easiest example of that, so everyone has bought on to the network standardization of the process, but each of the systems have different ideas as to what types of patient-reported outcomes they can collect with this new system that they think is really cool and could really save a lot of their time and workflow. So, one group might want a quality of life scale, another group might want a BHQ9, and so what we're trying to do is say, all right, well, from PCORnet we're beholden to collect these patient-reported outcomes.

We want the survey done in two to three minutes. What could you add on top of this that could be of benefit to you so that we can offer some type of customization and really meet the clinical decision makers in the middle of -- the intersection between quality improvement and research?

This slide outlines a number of our partnerships, both across CDRNs and PPRNs and then also outside of the network and what we're working

on. So, I mentioned the global patient IT. We're also working with CAPriCORN and pSCANNER on VA engagement specifically VA data contributions and there's a national workgroup on that. And then two of the other CDRNs have sickle cell cohorts and two of the other CDRNs have diabetes cohorts, so our researchers and staff have met regularly to coordinate research interests.

From a PPRN point of view we're currently working with two with the goal of eventually creating frameworks and templates that then we can use to engage other PPRNs. So, the Healthy Heart Alliance, we're working with Patient Engagement and CONNECT PPRN. We are running queries for them, and again, the goal is to have these as our initial relationships and then expand out from there.

Potential network members, we are working with our local VA office as well. Conveniently, they're in the same office building as me. From a health informatics point of view we're working with the Louisiana Optical Network Initiative, which is just a high speed broadband line that a number of

cancer researchers and other researchers across the state have used to work with genetic data and other big data initiatives.

Also, both Tulane University -- both LSU and Ochsner received an NCI community oncology research program and so we're working with them and, in fact, other research that's going on across the state to really be able to leverage the research that's going on across the state with the Louisiana CDRN.

So, finally, I'll end with a vision in impact. I mean, there's a lot of talk in PCORI and IOM circles about this learning health system and given that we are a number of health systems in a state, we're really starting to talk about this as a learning health state, and the goals are to create capacity, to embed these pragmatic research within health systems, develop an innovative patient engagement approach through our app suite and Health in Our Hands patient network, leverage resources through collaborations with other statewide research projects and CDRNs and PPRNs, as

was demonstrated in that last slide, and build and maintain a standardized and valid date infrastructure.

So, once again, thank you for your time and I'm going to pass it over to Rachel.

DR. HESS: Thanks Tom. I script it and so they've generously, now I have like multiple devices to advance, so if things don't quite work right, just holler and say you forgot to move the slide up there, and I was just thinking about what Tom said, trying to get surveys down to two to three minutes, we've got -- we're trying to convince investigators that they don't need 40-minute patient reported outcome surveys and that like seven minutes is about what you can do. It's like, no, it's seven minutes. I was like, try it with patients. They're shocked.

Anyway, so, PaTH is joining together the University of Pittsburgh, the University of Pittsburgh Medical Center, Penn State Hershey and Penn State Hershey College of Medicine, the Temple University School of Medicine and the Johns Hopkins

University and their health system to form PaTH. We really care together in response to the PCORI CDRN applications but had been joining collaborations that were both regional, that were loosely affiliated on other projects, and that were personal, all the PIs of our networks are general interest, so we've known each other from many different spaces.

The University of Pittsburgh Comparative Effectiveness Research core serves as our data hub for PaTH and the PCORnet CDRN Coordinating Center serves as the data hub for the PCORnet CDRNs and PPRNs to unify data there.

In PCORnet, each of the 11 clinical data research networks were asked to identify two unique cohorts and one common cohort of patients to survey with one rare condition and one common condition. In PaTH we chose idiopathic pulmonary fibrosis as our rare condition. For those of you that don't know, IPF is an insidious disease that attacks the air sacks of the lungs and results in scar tissue formation, which leads to a fairly slow and

difficult death with breathing becoming more and more difficult. It affects about 130,000 individuals in the United States.

Our common condition is atrial fibrillation, or a-fib. It's an anomalous sort of heartbeat in which -- heart rhythm in which patients develop fatigue and are at high risk for stroke and other embolic events due to the heart not beating right and the blood clotting within the heart and becoming more firm in that way.

And our third condition was obesity, because everyone's condition was obesity, but we have really focused on the long-term outcomes of bariatric surgery. While about 200,000 each year have bariatric surgery, that's only an estimated 1 percent of the U.S. population that's eligible for bariatric surgery, which is kind of a shockingly awful number, but a treatment that is underutilized.

We've partnered with our patient stakeholders and I think that IPF really presents a great exemplar of the way that we've partnered with

those stakeholders. We've worked with support groups and other patient advocacy groups within our institutions and really tried to talk to them about what's important in patient-centered outcomes, and the funny story around all of that is that we took our IPF questionnaires to our patients and they said, these are great, but what people don't ask us about is intimacy. Nobody asks us about intimacy. People never consider how this disease affects our intimate relationships with our partners.

So, we found what we thought was what they were talking about and so we went to the promised measures and looked at their sexual functioning questionnaire, we brought that back, and they're like, whoa, that was more intimacy than we were talking about. We wanted a little less intimacy. We were like, okay, we can find a middle ground between no intimacy and a lot of intimacy.

So, when we began to develop PaTH, we, working with our clinician stakeholders and our patient stakeholders, we created these three avatars that we -- that have really guided our

implementation of this network and keep us focused on a day-to-day basis of what we're doing, and I'll just tell you a little bit about them. We named them. So, Fred is our 67-year-old gentleman who has progressive shortness of breath and was just diagnosed with IPF and he is really struggling with what the next phase in all of this is. Does he need lung transplantation? What's going to happen with oxygen therapy? And what does this prognosis mean for his family?

Manuel is a 50-year-old man who was recently diagnosed with a-fib and whose travel schedule makes it very difficult for him to get the blood work necessary to manage his anti-coagulation therapy to prevent him from getting a stroke, and the medication he's taking to control his heart rate makes him feel fatigued, and that's not really working for him.

And Myrtle is our 60-year-old woman with obesity and she's struggled with her weight her whole life. She exercises regularly and eats well but can't lose weight. I think we all know Myrtle.

At her last visit, her doctor told her she was developing diabetes and between that and trying to keep up with her young grandchildren with her knee osteoarthritis, she's considering the Al Roker surgery but has questions.

So, these are the people that we try and bring this back to. These are sort of our touchstones that we come to in PaTH.

We were asked to talk about sort of what our model was. We've really struggled and, I think, achieved a collaborative decision-making model. For those of you that have tried to institute collaborative decision-making in an unwieldy group of four high-powered institutions with four high-powered PIs, all of whom have very strong opinions, you may think that that might be as overly ambitious as PCORnet, but I think we've done it pretty well.

So, what that means, we communicate frequently, we have had, since May of 2013, weekly steering committee phone calls amongst the PIs and now the project managers. In addition to that, we

have about seven committees that meet on a weekly to bi-weekly basis and we trust each other that we're not all on all eight of those phone calls every week. We've really created a shared leadership. Each of those groups is chaired by a different investigator and managed by a different project manager.

We make decisions, we make them by consensus and when those consensus decisions are not able to be made, we're very explicit about why the consensus -- why we need to make a decision and we meet in person. We're going into our second in-person meeting, which may not be as frequent as they should be, but that's on Thursday. We're really excited.

And we've done this not because it's the most efficient way to run the 18-month PCORnet CDRN project. It's clearly not the most efficient way to run the PCORnet CDRN project, but we've done it that way because we think that PCORnet and what it represents and what you as the Board of Governors have asked it to represent was more than the 18

months or more than Phase II, but really trying to create a new way of doing things, and we felt very strongly that all of the institutions involved had to be as invested in it as any single institution or any primary institution and that everyone needed to be invested in this idea of a network.

And so it may not be the fastest way to do now, but we think it's the best way to do the future.

So, what have our successes been? Our first six months have really been met with many successes and many challenges. In terms of data harmonization, we have met the goals of bringing our own data together as well as bringing our data into PCORnet and we're really excited about that. We've gotten our four institutions to agree to a single IRB model, which sounds like -- it either sounds really easy or like total miraculousness. I think it's total miraculousness, but we've created this thing called the PaTH Network Protocol Review Committee, which has IRB representatives and patient representatives from each of our four

institutions that act as the review committee for our IRBs and then Hopkins as the IRB of record.

So, we've managed to continue our distributive model of decision-making while creating something that can hopefully function a little bit more efficiently than that.

We've generated a lot of excitement about using PaTH. I'd say that at least once a week if not more frequently, one of us has a query from an investigator at our institution or from another site about using PaTH and how can PaTH be used to further their research. And we usually say, it's not just PaTH, you can use PCORnet too. And we've collaborated with network partners.

We have been able to work with other CDRNs, to explore multi-CDRN studies, and we have a junior investigator in our institution who studies sickle cell disease and we've been able to hook him up with the sickle cell group in Louisiana and throughout PCORnet to actually move his research along faster than it ever would have been moved along were he a single junior investigator at the

University of Pittsburgh, which I think is super special.

We also have challenges. I put complete data up there as the first of our challenges because I think that, in some ways, for me, is a little bit of the elephant in the room. We're trying to define complete data and in doing so to say, you know, do we know everything about this person.

We're using insurance claims data to back in and that means that we are probably under representing the underrepresented minorities, working poor within our network in that way. We have challenges fully integrating patients into governance. We have way too many meetings every week and we're integrating people in as best and as quickly as we can, but not as quickly as I would have liked.

We've engaged people in decision-making but I don't know that we've fully achieved that integrated partnership that I think is necessary and so we continue to work through those in the

next 12 months of this award.

We're having trouble with sustainability in the cost structures of that. I'm not a cost accountant. We don't have cost accountants on our teams. And figuring out what it takes to make a widget or a data element or anything else is a lot, and we're doing it and we're going to make it through and we're engaging with the people at our institutions that do that, but it's definitely something that I was like, whoa, that wasn't what I was prepared for.

And then timeline, I think Joe said it best, one day of PCORnet time is like four days of reality, so, we just keep saying that, and I say "I'm sorry" a lot to a lot of people.

We found that new partnerships in our journey, our local network is new partnership, and it's a great partnership, our patient stakeholders are new partnerships, our clinical stakeholders, we've brought together experts in each of our disease states from each of our institutions, some of them had worked together before, others hadn't.

They are so excited about the collaborative research that they're moving forward. And then our national network, I think that we all would agree that we've met people that we never thought we would meet or work with and have formed great relationships in that way.

So, clearly, there are problems beyond medicine, but in medicine I think the problem that we talk about and the problem that PCORnet is set up to solve is this idea of delivering the right care for every patient. That's our goal, that every person come out of healthcare healthier than they were and not less healthy -- healthier than they were, as healthy as they can be, and not less healthy than they were before they went in. Sometimes we don't know what the data are, sometimes we don't know what the right answer is, and PCORnet's really strong in that.

PCORnet gives us the opportunity to use not only collected -- data that was collected for clinical care, but also data that we gather in pragmatic clinical trials to find those answers.

We then struggle with adoption, how do we get people to get the right thing done in under seven years or under 14 years, whatever the latest number is, and then how -- once they believe it, how do we make it easy to do the right thing instead of easier to do the wrong thing?

So, this is my obligatory talk New Yorker cartoon, which is, we're ready to begin the next phase of keeping things exactly the way they are, and that is not where PCORnet is. So, PCORnet means to me that we're changing the way that we generate data and implement findings to improve health and quality of life, and in this process, we really run the risk of being overly ambitious. We can try and do too much too fast, and incremental progress is important and should be valued, as long as the focus is on making sure that the culture has changed.

PCORnet's in its initial phase on focusing to ensure that the infrastructure is in place to generate data more efficiently than before and as we look forward past Phase I, we will be able to

focus on implementation strategies. And this is our first meeting in November of 2013, you'll notice that that's before the awards were made. We were an ambitious network and decided that we needed to keep working because we would never finish in 18 months if we didn't, so this is Penn State Hershey Medical Center and this is our first fall meeting when it was just a bunch of people traveling on their own dime to there and we had people from every institution, which was great, and this is our logo, this is PaTH Network for Patient Empowered Research.

Thank you very much.

CHAIRMAN NORQUIST: Thanks. We're going to have to move you guys along a little bit here because we're going to run out of time.

DR. HESS: Oh, sorry.

CHAIRMAN NORQUIST: It's okay. I know I -- can we break at this point, Rachel, because now we have the PPRNs, and maybe we want to have some questions specifically on the CDRNs first? So, we may break at this point just a second. We'll let

you guys prepare your talk while we're doing this.

So, let me go -- we're just going to go around. So, Steve, you're up first.

VICE CHAIRMAN LIPSTEIN: You said something that triggered --

CHAIRMAN NORQUIST: Steve Lipstein.

VICE CHAIRMAN LIPSTEIN: Steve Lipstein, member of the Board. You said something that kind of triggered almost an alarm with me and I wanted you to go back and explain it. You said that you were going to use claims data for something. Do you remember the remark you made about claims data? And one of the concerns at least the constituency that I represent on this Board has, is that claims data is just not sufficient to the task of outcomes research.

DR. HESS: Oh, I'm sorry -- yeah, yeah, yeah. I should have said that more completely. In order to make sure that we can understand complete data from our EHRs, we are backing that up with claims data.

VICE CHAIRMAN LIPSTEIN: What does that

mean?

DR. HESS: It means that -- so, for us what that means is that we've created a strategy for defining a complete patient and it's a multi-pronged approach. The first is, does this patient have data within the care network that we represent? So, I can go through the example probably most clearly for the University of Pittsburgh and UPMC that UPMC and UPMC Health Plan covers somewhere between 300,000 and 400,000 people that they see together, meaning that they both are insured by the health plan and they are seen by the medical center.

For the majority of those people, over 50 percent of the care that they received is received at UPMC Medical Center, but they may travel and see something that may relate to one of the diseases or conditions that we're studying that happens outside of the medical center and given the lack of national patient identifying data within the country that we live, we don't necessarily have the cleanest way to find out about that data beyond

patient surveys, so then we're using claims data to back up what happens outside of our institutions.

VICE CHAIRMAN LIPSTEIN: Right. So, what you're saying is using claims data to be additive to?

DR. HESS: Yes. Yes.

VICE CHAIRMAN LIPSTEIN: Good. That helps me.

DR. HESS: Okay, sorry.

CHAIRMAN NORQUIST: Next is Bob? Okay.

MS. SIGAL: Ellen Sigal. Board. I have a question, Tom, for you. Great presentation. Really interesting. So, you have a lot of money from NIH. Do you have any state money at all for what you're doing? Is there the state --

MR. CARTON: No. I'm glad you asked that though. One of the things that we're getting ready to do is to be able to have discussions with our state economic development office and start to explain not just the power of this PCORnet, but also it's one of the reasons why we want to integrate with NCORP, LA CaTS, other research

projects that are going on across the state. It's in our planning. We've laid the groundwork for those conversations.

MS. SIGAL: I'm meeting with your governor next week, maybe we can talk about it.

MR. CARTON: That would be great. Thank you.

CHAIRMAN NORQUIST: Okay, Bob?

DR. ZWOLAK: Bob Zwolak, governor. Mr. Carton, you made a relatively strong point about assigning an individual patient identifier and justification patients moving between hospital systems, hospital systems changing, are the other CDRNs doing that? And are all the CDRNs together trying to collaborate so that you would have a universal patient identifier across all CDRNs?

MR. CARTON: Other CDRNs are working on it as the slide that I put up in terms of the collaboration. It's really CAPriCORN, Chicago CDRN, that is leading this charge. They're the ones that created the hash algorithm, it's an open-source software that they're going to make

available to us and to other CDRNs.

We, as a small working group, have talked about bringing that idea to PCORnet across the board and just haven't as of yet. Like I said, I don't want to speak too much about it, it's really the Chicago piece and we're collaborating with them, but I think that there is an opportunity there.

I can also say that our systems were very skeptical in participating in the de-duplication process if PHI was involved, so the solution is very powerful in that it doesn't involve PHI being stored in one particular place.

It is our plan for working with claims data as well when we can talk to holders of claims data to explain that the data can be shared with the network without sharing any PHI.

So, it's -- the question is kind of beyond me, but I think that it's worthwhile to pursue.

CHAIRMAN NORQUIST: Sharon. Before we start putting up a lot of tent cards, I want to give the PPRNs a chance to also have their say

here, so go ahead.

DR. LEVINE: This will be brief. Rachel, just a quick question for you. Looking at your partners, they're all academic institutions, and I guess for me the big question is, why Geisinger was not part of your network given the --

DR. HESS: Do you want the real answer?

DR. LEVINE: Yes.

DR. HESS: We actually asked Geisinger and the University of Pennsylvania to join the network and they said that they were doing their own network and we could join their network, and I said, but we're pretty far along on our network, and they said, no thank you.

DR. LEVINE: Thank you.

DR. HESS: Can I back up that with just saying that actually all of our health systems also have, in the 2000s and before that, actively engaged in the mad dash to buy every community practice in their purview, and so we have a lot more community practices than one would think looking at the health system.

CHAIRMAN NORQUIST: Thanks. Rick?

DR. KUNTZ: Rick Kuntz, Board member. First of all, thanks for your presentations and coming out here and want to congratulate your pioneering this area.

I'm still a little confused about your data structure. You know, networks that generally have parallel research systems put a lot of attention on things like completeness of ascertainment, standardization of outcomes and so on, but it seems -- are you leveraging EHRs and claims data together as -- I mean, how are you identifying the quality of the data to be reliable enough to actually publish at this transition area? Because, as you know, the Holy Grail is trying to understand how to take practice data and repurpose it for research, and is that a goal of yours or do you have that solved already?

DR. HESS: It is a goal. We don't have it solved. I think that hits to my point that PCORnet and its mission is extremely ambitious and I don't know that we will have completely solved that

problem by the end of 18 months, but what I hope and what I believe very strongly is that we're progressing on that continuum to solving that problem and are committed -- I mean, the informatics brains in this group -- I am not an informatician, and I don't pretend to be one -- they're crazy smart. Like, we have the best people in the entire country, maybe the world, as far as I can tell, working on this and desperately trying to move it forward, and I have no hesitation that we'll get there. We may not be there by September 2015, but we'll be closer.

MS. FLEURENCE: I can add to that. Our coordinating center is working on that and they do have protocols ready to start doing what they call basic characterization, so they'll be looking very closely at that as well in addition to what the networks are doing.

CHAIRMAN NORQUIST: Thanks. Larry, you've got the last --

MR. BECKER: So, State of Pennsylvania, the State of Louisiana, maybe a little more than

Pennsylvania, and you refer to it in PHI, you refer to it in the Geisinger question, but you got systems that I think probably are competitive to work together. So, could you comment on what keys you used to get the competitors to work together where you were able to do that?

MR. CARTON: Well, I think the power of PCORI and PCORnet did a lot of the heavy lifting for us. People are -- the networks and the systems, even before we were awarded, came to Louisiana Public Health Institute and really expressed interest in participating in this network for the vision and the downstream health systems change that's in their personal interest of delivering care and being research institutions.

As a public health institute, we're uniquely positioned in that we're not one of those competitors, and so we're drawing people to the table, but really that sell of participation and joining was one of the easiest. The more difficult ones are global patient -- the details once they've agreed, once the agreement is there, okay? How

deduplify patients, how to create an EMR agnostic engagement system and suite, so Rachel might have a different play on it. I mean, we're a bit unique in that we're a public health institute that's kind of outside of that competition ourselves.

CHAIRMAN NORQUIST: Harlan, if you promise to make your --

DR. KRUMHOLZ: Yeah, and I just wanted to -- Harlan Krumholz from the Board, and really impressive the speed with which you're learning and the ground you're covering and the tools that you're developing.

I wonder if you would be willing to work with us to develop an ongoing blog that's almost on a weekly basis of what you guys are dealing with and what kind of solutions you're coming up with so that people around the country can track with you to potentially crowd-source some of the solutions, share some of the tools with the smartest people on earth, there are other smartest people on earth, you know, create different streams for the informaticists, for the clinicians, for the

patients, so that this can be -- we would have to invest in it so it didn't burden you with this, but almost taking what's going on in real time and creating an ongoing blog about it that could then be leveraged with national learning.

Because it just seems to me like there's so much here, and in some ways it's been sequestered, and you guys are so damn busy you don't have time just to do this documentation, so if we could provide -- would you be averse to that or would that be something you think would be a good idea?

DR. HESS: [Off microphone.]

CHAIRMAN NORQUIST: She got a little pale there, I thought --

DR. HESS: So, I would say two things, one, yeah, that sounds really great. The second -- the second is that I almost wonder, as we're -- so, one of the things that we are doing contractually is that, I think, it's like 100, we have like 100-something deliverable documents that we're submitting; I'm behind on those, I apologize, but

I'm almost wondering if some of those documents could be -- like, that stuff could be extracted from those and shared and that might be a way to kind of convey that in real time?

CHAIRMAN NORQUIST: Let me -- we're going to need to move on, but I think one of the things we could do on that -- it's an excellent recommendation, we can certainly have our communications director, Bill, work with you guys and think up a way on that, certainly through the RTC, you guys --

MR. SILBERG: Yes. I just want to say that the RTC is considering communications [off microphone].

CHAIRMAN NORQUIST: We'll pull it back in with our special Board committee that will focus on that and then we'll have our communications director work with them. Okay, we'll let the PPRNs have their day also.

MS. PEAY: Great. Well, I'm Holly Peay. I'm very excited to tell you about the PPRN that we're running, which is called DuchenneConnect.

DuchenneConnect is a patient and family self-report registry for Duchenne and Becker muscular dystrophy. DuchenneConnect has been around since 2007. It is a project of Parent Project Muscular Dystrophy, which is the foundation that is run by parents and families of individuals with Duchenne and Becker muscular dystrophy.

We have, at this point, about 3,000 registrants in DuchenneConnect, so we had existing data as we came in to PCORnet and we have a very extensive survey that we ask people to fill out.

Let me give you a little background about Duchenne and Becker and then I'll tell you about some of the data we collect.

So, Duchenne muscular dystrophy is the most common, fatal, neuromuscular disease of childhood. It is still a rare disease but it has about 13 to 15 thousand individuals in the United States who are affected. We actually don't know exactly what the prevalence is, but hopefully we will soon.

The way that we use DuchenneConnect is to

help speed research. So, the intent about this being developed through the community, through a parent foundation, was that research was taking too long and that was both academic-based research and research that was done through industry sponsors, and so DuchenneConnect is intended to speed up the proctor research process, the recruitment process, and really make research happen much more quickly.

So, we had some of the same goals of PCORnet going in, which was very nice for us, so I'm going to talk to you about the PCORnet kinds of initiatives we're doing through DuchenneConnect, but we have a broader set of initiatives as well that is going out to the community.

So, the DuchenneConnect history I told you a little bit about. I will tell you a little bit more about how we actually use the data. So, we do have a history, again, of having full commitment, an enormous amount of clinical data, and what we've done to date is had that data curated. So, we actually have patient-reported data that we then ask people to submit different kinds of lab reports

and then those are curated by a professional so that people who are using our data, it's one of the common questions is how do we know that people put in the right data. I'm glad to say, actually, peoples' self-submission is very good; it's very consistent with what we get from their clinical reports.

And one of the very nice things about our platform is we collaborated from the inception of the registry with a group called Patient Crossroads, and I'll tell you a little bit more about them, but they do our platform and they've been able to use the platform we created actually to support more than 200 other, mostly rare disease registries.

So, PCORnet dollars are similarly being amplified because of what Patient Crossroads does with us through the project they can similarly extend to these hundreds of other patient groups under their network, so you get a nice spread of your dollars that way.

So, our team includes Patient Crossroads,

like I said. UCLA is one of our collaborators, Geisinger Health Systems is one of our collaborators, and we have a leadership committee, and I'll talk to you more about them in a moment.

So, UCLA is predominantly working with us on changing our surveys. We want to make our survey more responsive to doing comparative effectiveness research, and they're also working with us on trying to identify more representative sample of the population. Our existing registry is very diverse in terms of clinical orientation. We're very consistent with natural history sorts of data, but we don't have good demographic representation, and we're trying to work with UCLA to do that.

Geisinger we're working with predominantly to try to integrate EHR data into our registry, so there's a lot of burden on our participants, we ask a lot of them, and we'd like to be able to minimize their burden and get very high quality data by pulling data directly from the electronic health system into our registry. And you all know about

Geisinger, so they're a good partner with us to work on this and they're very committed to this, it's quite nice.

So, how do we work together? So, you know, we have, again, these academic groups working together with a patient powered network, working together with a patient leadership committee, so we really do every decision together, and here's one example. So, we were looking at integrating some new patient reported outcome measures into our registry and this is something you all may be familiar with, this is the Neuro-QOL, and this is looking at lower extremity functions, so this is one of the Promise offshoots.

And so the researchers were very interested, said, this is perfect, this is validated, this is great. We asked the parents and the patients, our leadership committee, and they said, hmm, we're not going to answer this the way you want us to. So, it doesn't say can you do it by yourself, it doesn't give any parameters on how this is done. So, for example, can you get up and

down a curb?

So we have many adults who are in wheel chairs and they say, yeah, I carry a ramp with me and I get it out of the back and it takes 20 minutes, but I can do it. I do it with maybe a little difficulty. Okay?

So, that kind of engagement has really led us to change the way we're approaching the questions we're putting in our registry and that's one example of the way that our team works together.

So, we've been very -- we call it a family center instead of a patient center because this is primarily a pediatric disease or onset in pediatric years. A lot of our advisors are patients and a lot of our advisors are parents. So, we talk about family-center governance.

We have historical engagement. When the registry was created, we had an advisory board and they still are with us today, and that included the whole community, so researchers, all of our users as well as families. We also then have our PCORnet

leadership committee and they do lots of work for us. Really, they're involved in every component of this project. And one other example of how we use them is we've engaged them about identifying research priorities.

It's a real challenge to talk to parents and individuals managing Duchenne, which is a very burdensome illness, and to say to them, what are your research priorities, and they look at you like, seriously. So, we've worked really carefully with our leadership committee to come up with a five-step process to engage them, to develop a research imagination in these families, and to do it in a way that doesn't feel like another thing they have to manage.

So, we're engaging them around what are the important things for you and for your family. We have to look at different stages because the stages of illness have very different needs. And they've walked us really nicely through how we do that engagement. It's been really helpful.

We also have PCORnet partnerships, and I'm

going to mention, really, just one of them now. Several of us use what's called UC-REX, this is a sidecar, a data sidecar from the California system, and there's a very nice, big set of data sitting there.

So, when we made this collaboration with UCLA, we said, you know, it would be really nice if we could talk to those people in that system and let them know about DuchenneConnect and invite them in. They have a very nice diversity of patients there.

And so it turned out, low and behold, lots of us are using UC-REX so we all got together and you can see the lift here, and we started talking about doing this together. So, we're doing a joint IRB proposal with all of our projects together, and we're also working together on the possibility of actually pulling data from that UC-REX system into our respective registries.

So, I'll just sort of wrap up by talking about some of our challenges and successes in the first six months. So, one of the challenges has

been around community engagement. So, I already talked to you a little about our community.

They are managing really serious illness with a fatal endpoint and so when you talk to them about a very technical infrastructure project, what they say is, how does this help me now? So, we say, really, it's not helping you now except for some of the additional add-ons that we are able to do to our registry, but the long -- in their world, for us it feels very fast, for our families it feels very long. So, it's a little bit of a challenge to engage them around this.

So, we have had a very successful leadership committee that's really helping us with that. From the inception of our registry, we've been very committed to giving back, so we don't ask for any data without giving something back to people. We have been able to really push on that. Historically, the way we've mostly given back is by providing answers. So, you say this about your kid, here's what everyone else said. But now we're able to get some much more rich data back to people

and education materials back.

Rare disease has its own set of challenges. Some of our priorities kind of get lost in common disease. Diversity is challenging, and also small numbers, if you have a small population, you really need them to get engaged and to stay engaged and it's a challenge, but we do also have highly altruistic parents and patients who tend to have fewer privacy concerns, and I say this with some data behind it.

So, the final challenge is that there are so many possibilities and there's so little time. It is so exciting to be part of this network, but it is a stretch and especially for a small foundation, this has really been overwhelming to our staff, but overwhelming in a very positive way because there are so many things we can do.

And I'll end with our vision. So, we really hope in the next 12 months to continue to act as good citizens as PCORnet. It's easy to get mired down in your own network, but we are hoping to continue to rise above it and think about

PCORnet as a whole network; increasing awareness of Duchenne and Becker muscular dystrophy, increasing the community empowerment; and really, our main target here is using the power of this to answer questions that are important to our families. So, we want to get the most out of Phase I and make sure we're involved in Phase II so that these very basic questions and the more complex care questions are things that we can actually answer through the network.

Thank you.

[Off microphone discussion.]

CHAIRMAN NORQUIST: That's all right. We can go into the next --

MS. TERRY: Okay. My name is Sharon Terry and I'm the president and CEO of Genetic Alliance.

So, background on Community Engaged Network for All, Genetic Alliance is an advocacy umbrella, we're the lead organization for this project. There are nine disease advocacy organizations, which we chose from dozens of applicants, during the process of bringing the

application together and you can see them listed there, so I'm not going to read them all, they're both common, chronic conditions as well as rare diseases; two universities, University of California, San Francisco and Davis; and one technology partner, Private Access.

Our governance is Disease Advocacy Organization Leadership and Patient Council, so this is completely lay run. It is all patients, all participants, all the time. Our planning has included building standards and common data elements and instruments obviously using ones like Promise and Phoenix, et cetera, that are already available, but also looking at how to build those as we move forward, community consultations times nine, because in everything we do, every community is involved, and then best practices for guides and navigators, which I'll talk about in a second, and outreach in general.

For implementation and testing, the executive committee, which is comprised of the genetic alliance, private access, and the two UCs

really manage those pieces. We have nine launches, two of them occurred today, so hopefully you'll see that press release for Gaucher and for Joubert, and then outreach, dozens of communities and partners all working in concert with one another.

We've been working on computable phenotypes, which is a nickname that some of us don't particularly care for, looking how can we use things like electronic capture systems to look for these phenotypes that are actually quite complicated and don't have, for example, ICD9 or SNOMED codes. We are working on crowd source proposals with something called Open Proposal, that the University of California, San Francisco has piloted in the university. Essentially each of the disease groups will put up a proposal, the community and more, because it's open, totally open, can then refine that proposal in the public eye, so it's a crowd source kind of platform.

And then analysis, we hope to engage in before the end of the 12 months, again, using things like Promise 29 and quality of life

measures, sometimes just across our nine and sometimes across PCORnet in the places that we're all using those.

So, the platform we're using is called the Platform for Engaging Everyone Responsibly. I'm showing you here the Joubert site that launched today, in fact, multiple guides from the community engage the community. We believe that the one common element in all these problems about trying to figure out what is complete data and where is someone's data and how do you figure out how to reduce duplication is the individual. That is the one row across all the columns, and if that individual is in charge of saying where their data should go, then that individual can really help us solve those problems.

These guides, then, help individuals make decisions about where their data will go, and those individuals are looking at things like in multiple categories, advocacy organizations, researchers, data analysis platforms, am I going to say "allow", am I going to say "ask me", which is a kind of

maybe that reduces to the binary-ness of the system, or am I going to say “deny”? I’m going to say that about three different sets of data: discover me, which is anonymous data, export and use my data, which is actual transaction with the data itself, and contact me so that I can participate in other trials, et cetera.

We found, so far, about 85 percent of individuals say “allow, allow, allow”, 10 percent say “ask me”, and 5 percent say “deny”.

Participants can set these themselves using the guides as their models and about 85 percent use the guide.

The participants get back immediate feedback. So, unlike the survey fatigue we see in other instances, we’re finding individuals are asking -- answering a total of 200 questions in a sitting. They can log back in and answer more questions. The system has about 26,000 questions in this “gameified” manner so far, and each of these disease communities are adding more, again, trying to constrain them as much as they can to

common data elements and already validated instruments.

Our successes are that all these organizations, which some people will tell you, well, you know, the nonprofit community is fabulous because we always collaborate and cooperate, I would rather work with pharmaceutical companies and the government than nonprofits sometimes because we can be as cutthroat as the next.

So, for these nine organizations to come together and work together this well and meet all of our milestones, I think, has been quite incredible. That we've been able to come together cross-condition and have consensus about what tools we will use together has been great.

Robust connections to the clinical community, which I'm not sure if PCORnet has across PCORnet, but because each of these organizations has between 10 and 100 clinical advisors, there's lots of clinical, community involvement, innovative engagement with the two UCs, who are very excited to be doing this with us, and then increased

literacy overall in the advocacy community around what is a common data element, what's a valid instrument.

And then I think our challenges are scope creep, I like to say to the group that we assembled, we said we would use a bicycle for our registry system and we've instead delivered a Tesla, and it's pretty amazing, but it's just every new thing is important and we discover things that we should add and we do; diversity, I think diversity is a crisis for PCORnet and for the nation. I don't think we're taking it as seriously as we need to and I know we care deeply about it, but I'm not sure that we have met that need the way we should; and then time, everybody's mentioned time a lot, I think I'm somewhere around 80 percent time for PCORnet and nowhere near paid that, but it's really been worth the investment overall.

We were asked to comment on patient engagement -- I like the word participant better than patient because we, like Holly, don't have patients, we have people who are affected by

diseases and don't consider themselves patients every minute of the day, and I'm just going to leave this phrase for that, nothing about us, without us. They basically are involved in absolutely everything, so I don't have to say what's the challenge of having them be engaged.

Our new partnerships, Holly's mentioned one, with pSCANNER, informally with lots of others. Outside PCORnet, lots of partnerships interested in the process, the policies, and interested in our particular platform. And finally, my vision, the vision for PCORnet, I think the first is that research is radically altered when we get to the end game, whether that's in 12 more months or five years, that people discover two secrets, my physician doesn't know everything, and I am essential to improving health in general, mine and others, that the public is part of innovations in health, that a learning healthcare system is powered by people -- I don't think we've gone far enough to actually use the tools that engage people in America and beyond -- and that health is

accessible to all. Thank you.

CHAIRMAN NORQUIST: Is that it? Thank you. So, we will now take questions, comments. So, Allen, since I cut you off the last time, now you can be first.

DR. DOUMA: I really want to thank all four of you for being here. It's amazing the amount of work you've done in such a short period of time and everyone ought to be proud, including PCORI staff, who have been the coordinators of you guys.

My concern is long-term, so I really want to focus a little bit on sustainability, but first let me remind myself, your comment about they're people, not patients, is my mantra, and I'm glad you said it, I think we need to think it, and sometimes I'm sorry the name of our organization is what it is. But that's another story.

With regard to sustainability, could you talk about the two steps, one is how do you get from where you are now to a few years from now so the infrastructure is fully solid, workable,

complete, et cetera, then the next step of doing your search. Can you talk about your thinking in terms of marketing yourselves to external organizations, whether they're governmental, non-governmental, or private sector, in order to sell the service that you're creating?

MS. TERRY: So, how do we get to the next step? So, for us it's been things like we have begun to talk to other foundations. Robert Wood Johnson, for example, has invited us to apply for a grant to white label this so that lots and lots of groups can use it. We've also looked at what can we do to have this be a sustainable endeavor, both from the disease advocacy organization's point of view, so perhaps they pay a very small fee. Perhaps we have an app store model, so that people are paying $1.99 for the service on the researcher end.

So, right now we're toying with a lot of business models.

On the how do we sell it to be essentially research ready? We're really hoping to test that

in the spring, and it is because each of these disease groups have ready communities that want access to data, want access to cohorts, we're looking at what can we say in terms of research readiness, what can we say in terms of PCOR that can already be done with the data that's coming in as well as bringing these people into the other projects in PCORnet, and we believe that if we set this ecosystem up right and we have catalogued this in such a way that it's accessible to the community, it will be available.

There's a couple ways to do that, one is we are attached as a common data model, and we'll be giving our data to that model in as much as individuals have said yes to that. The other is we've built a back end that's not quite finished called Recruit Source, that looks like the Google for health information, and researchers can Google it and find where is a cohort and how can I characterize that cohort to be ready to do a project.

CHAIRMAN NORQUIST: Anyone else? Harlan

Krumholz -- oh, did you want --

UNIDENTIFIED BOARD MEMBER: Yeah, I'd love to hear [off microphone] --

MS. PEAY: To tell you quickly how we've managed to sustain for seven years, so first of all, our organization has a very strong commitment because we are speeding research up and we do have data showing that we're doing that, and so the Parent Project Muscular Dystrophy is willing to continue to support the registry.

Second, industry is very willing to pay -- I mean, I think similar to Sharon, I know that making people pay for data is sort of a taboo in the advocacy world, but we do it all the time and industry is very willing to pay for good quality data and we have it. And so, we are sustainable because industry is going to pay for this and has paid for it.

CHAIRMAN NORQUIST: Actually, it's a very critical question, the whole sustainability across the board. So, thank you very much for that. Harlan?

DR. KRUMHOLZ: So, thank you all. I want to particularly -- just because we are now focusing on the PPRNs, thank Sharon and Holly. It's breathtaking, really, to think about the engagement.

I just wanted to say that at least when I first thought about this, I didn't think of it as being -- you being as comprehensively involved. I thought more of you funneling ideas and hiring people to pursue your ideas. You would be in charge, like you're at the top, you know, you're basically saying, we've got questions people aren't answering. We can bring people -- we need people to work for us to answer them, we need to control the data, and we need to make sure that it's honed in the right direction. And to me that seemed like enough, right, and you guys are actually taking on even more than that.

I mean, when you're selecting -- I thought it would be that these people would be working for you and you could look to PCORnet and say, hey, we've actually got -- people are funding us and

we've got some money, we're just looking for investigators to come on as partners, right, but we'll treat you nicely like you treated us before, but you know, we're all part of the same family now and there's a balance of power that didn't exist before.

I just wonder if you could reflect on -- have you taken this in a different direction -- which is fine, I'm just trying to understand it -- or do you see yourself -- I mean, I know the citizen science is something I know Sharon's talked a lot about, to me it was this combination more about giving you the power than it was about that you would have to do it all yourself and that you actually would be able to -- you know, the make/buy thing, you would be buying a lot. But what you're funneling is what uniquely you own, which is that is the ideas about what's important to people. And then also you could bring about the communities who are eager and ready to go.

If you did that to me, in my field, I mean, I would bow down before you because you're

making it easy for me. You're bringing the great questions, I can maybe contribute to them, and you're bringing me patients who are eager to be part of that.

Can you reflect back to us about how you are thinking about what the PPRNs are exactly?

DR. HESS: This is a very tough question. So, I think that the PPRNs -- the role of the PPRNs, as you've discussed, has, I think, merged over time, the thinking of how the PPRNs are involved in this network, and I really feel like that there are two ways -- one is the more typical idea of identifying major research questions, so I know what's important to my families because I ask and because they tell me where they might not tell their clinicians or -- you know, so we have a different relationship with them and we can collect different kinds of data. So, that's sort of the old school way of thinking about it.

We have a very engaged, committed cohort of people who want to be involved in their own research. So, we have a bunch of carrier moms who

have banded together and they're saying to us, you are going to address our needs and here's what we need and here's what we need you to do and how.

So, we say, okay, so we just started a whole new carrier project because they have very specific health questions and there was no data. Let's do it ourselves. So, I think there is a different feeling of empowerment and we can do this kind of research ourselves and we don't need a CDRN partner for everything.

Now, to get everything we need out of this network, we definitely do need the CDRN partners because that's where the real strength comes, but I think there is a growing feeling that we can do this. A lot of the questions are important to my community right now, they can identify themselves and we can answer.

MS. TERRY: I think the other thing for me that's so striking, Harlan, is that this -- that when PCORI was created, and thank you for the -- I mean, PCORnet was created, thank you for the idea, that the PPRNs were recognized at all remarkable.

It was -- you know, some of us had been doing this 20 years.

My kids were diagnosed in 1994 is when I left campus ministry to do this and suddenly somebody said, oh, you guys exist and now we're going to bless you and actually give you some money, which is like remarkable, I think one, there wasn't enough money for us to buy what we needed to buy. I just spent the weekend polishing CDs, you know, common data elements so they're nice and shiny so the launch could happen this morning. That's going to have to be part of it. We're going to have to roll up our sleeves and be digging in the dirt.

The second thing is, I love that we recognize PPRN. I don't think we've gone to the next step though and I think the next step is to say, and what are we not using that already exists in our culture that we all know is real easy in other cultures?

So, this morning on NPR I'm driving in and they say, electric cars are cool, how are you going

to make them cool enough for kids to buy? Well, you have a road race. It's happening in Beijing first and then it's coming to Miami and they didn't say, like, let's put out brochures and then let's educate and, you know, nobody's listening to that stuff.

So, I think until we, PCORI and PCORnet figure out, let's go a step further, it's still too medical model. I mean, Holly and I are really trying to stay out of the medical model, and we love our brother and sister CDRNs and we're working with them, but we and they know we need to go farther and I think that can be a really exciting next step.

CHAIRMAN NORQUIST: We need to -- okay, go ahead. Oh, it's not small, because the next part of this is something they're going to want to hear, which is the Phase II part of what we're proposing, so I want to be able to let -- Rachael, can you get to this, and then maybe we'll get to your question.

MS. FLEURENCE: Just a couple of important points so, I'm going to speed through the intro.

So, and you all have the slides and the slides are going to be available publicly as well, so just to start off by saying we are 12 months away from Phase II. On this slide you have the timeline of sort of the major achievements that we've had to date and we plan to make an announcement for Phase II in December and the Board will have to approve the RFP, but this is sort of the initial information that we want to propose to you for discussion around Phase II and it's been reviewed several times by the RTC and approved.

So, I'm going to skip over 18-month aim and four-year aim. They're in your slides and you're familiar with these at this point. And so what we want to get to is back to Gray's question and Allen's question about sustainability, what we're proposing for Phase II.

So, essentially we see a number of ways PCORnet can be sustained. The one I'm going to focus on today is an infrastructure investment from PCORI, it will be a tapering infrastructure investment, but we do expect that other sources

will be available, such as competitive funding through PCORI's general funding announcements as well as research funding from other institutions, such as NIH and potentially industry.

I'm going to focus on the tapering infrastructure funding today.

We went through an RFI process in order to be able to really understand what the costs might be of sustaining an infrastructure like PCORnet and we essentially decided that this tapering infrastructure investment that we're going to propose to you has two components, one is around the organizational sustainability and the second is around the data sustainability.

Other needs of the networks include funding for actual research projects, which we see as separate, and the networks will have to compete for that kind of funding, as well as pilot work, so innovative kind of work but it's not the full CER type proposal but that might be other kinds of pilot work.

So, that's not the subject today. Today

we're going to talk to you about both the organizational and the data sustainability that we are proposing for the Phase II infrastructure funding.

The next two slides, and I'm not going to go through these in the interest of time, since we're a little behind, I've described the types of activities that we think both the CDRNs and the PPRNs will be undertaking with this infrastructure funding. They relate to some organizational maintenance, so how you maintain a structure like the ones that you heard about today, and then there's quite a bit of investment needed for the data maintenance, and we talked about data quality, for example, there's quite a lot of work that goes into refreshing your data, checking your data, continuing to harmonize it to the common data model.

So, these are some of the activities we see for the infrastructure portion of the CDRN investments. Very similar types of activities for the PPRNs, I won't read through them again, but

again, they have organizational maintenance requirements as well as data maintenance requirements.

So, this is where I catch my breath and I'll spend a little bit more time on this slide and the next. So, there's a couple of important points that we would like to make about Phase II. The first one is that this is going to go through a request for proposal mechanism, so an RFP mechanism, that's a little different from how we did Phase I. It will be a clearly specified statement of work, and this is essentially because we're mid project and it makes much more sense to go through that kind of mechanism than through the other mechanism that we used for Phase I.

We are expecting that funding will be available for up to 13 CDRNs and 22 PPRNs. As a reminder, we currently have 11 CDRNs and 18 PPRNs, so we're leaving the door open for a few new organizations to enter, but we also want to remind our current awardees that it is a competitive refund, so they won't automatically be refunded for

Phase II. But these are our total numbers proposed as of to date.

A few things about the CDRN requirements that we'll be having in Phase II, we are going to be requiring that a clinical researcher be either the principle investigator or the co-principle investigator of the CDRN and we'll be asking for specific time commitments to PCORnet. I mean, we heard from our PIs today, PCORnet has really been a huge time investment for everyone involved and sort of really enabling our PIs to have the amount of commitment that they need is going to be important.

We're going to continue to ask them to describe their clinical areas of expertise. They each have -- each CDRNs have three cohorts in which they have expertise. We're going to really ask them to show progress in these areas and to show sort of finds of research application that they'll put forward in these areas, and so we really want to see -- as far as seeing the signs of research readiness for Phase II, particularly linked to the cohorts.

We are very intent to work with NCATS on the CTSA front, so it's going to be very critical for our CDRNs, where applicable, to be working very closely with their institutional CTSAs. So, the CTSA program, as you know, is undergoing some changes right now and we want to be leveraging these changes and working very closely with the CTSAs so that we're really making good use of the investments that NCATS is making as well.

And then a final point here is just this continued focus on achieving data completeness, and we're going to be making a renewed effort to ask our CDRNs to link with their health plans so that we can continue to solve some of these problems around data completeness.

For CDRNs and PPRNs, we're asking them to provide a sustainability plan that will start in year three, which is the year where we taper off the infrastructure funding from PCORI, as well as to show their cross-linkage plans, so a real increase in work between PPRN and CDRNs starting in Phase II.

This is the funding amount that we're proposing for CDRNs and PPRNs over this Phase II. It's a three-year program. The direct cost for CDRNs in years one and two would be $2.5 million with a tapering off in year three at $1.25 million for a three-year total of $6.25.

For PPRNs, it's $480,000 in year one and two, and $240,000 in year three for a total of $1.2 million for the three-year program. These numbers have been discussed by the RTC and they've been approved by the RTC in preparation for this Board meeting.

DR. DOUMA: Quick question? What are the present funding levels, annual funding levels?

MS. FLEURENCE: So, for the CDRNs, this represents -- so, annually equivalent this represents about 75 percent in years one and two, and then that decreases 50 percent in year three. For the PPRNs, it's slightly over what we're giving them today annually equivalent.

This is actually the end of my formal presentation, so we now have time for discussion.

So, these are the questions that we've teed up, so whether this description is consistent with your vision as the Board for PCORnet, and how do we leverage the capacity of the CDRNs and the PPRNs to do actual research?

DR. SELBY: And before we start, I just wanted to add one thing or just to emphasize one thing Rachael said, which is that we've made it clear, and we continue making it clear through the PCORnet's steering committee is that we expect that PCORnet will be research ready at the end of Phase I, that means either already actively engaged in research, perhaps in our aspirin trial, perhaps in analyses of the weight cohort or one of the network's cohorts, and very clear to us at the time of evaluation of applications that the CDRN or the PPRN is intent on both conducting clinical research, prepared to do it, prepared to collaborate across -- with other networks, so that will be one of the criteria that will be very closely adhered to in evaluating the applications for Phase II.

CHAIRMAN NORQUIST: Thanks, Joe. Before we go down the tent cards here, I wanted to let Freda, since you're the head of that strategy committee that oversees this, whether you wanted to add anything or say anything?

DR. LEWIS-HALL: No, I think -- let's let some of the questions go around.

CHAIRMAN NORQUIST: Alicia, since I cut you off at the -- you can --

AF: Actually, this was great to have the suggestions, but thank you so much for such interesting, stimulating presentations and particularly addressing to the PPRNs.

You both mentioned the issue of diversity and I wanted to have a little bit of -- give you a little bit of an opportunity to share with us some of your reflections and discussions on that issue, and the issue that I'm particularly -- some of the obvious ones that come to mind have to do with the clearly -- the nature of this requires a very high level of digital engagement, a high level of literacy, and I've wondered to what extent you have

engaged in discussions around ways of bridging that, be it with patient readers -- participant advocates, participant however you want to call it, participant enablers.

The second issue has to do with -- particularly, perhaps, Sharon, for you -- with the nine organizations that make up your -- to what extent is it not about the methods of collecting data but actually has to do with the makeup of your constituent organizations themselves and work that they may wish to do at their level rather than on the level of collecting the data?

So, if you could comment on that, and I think what would most interest me is your sense of where PCORI could be helpful, be it in setting a standard or be it in enabling you with resources to actually accomplish what you want, which is a data source and resource that is responsive to -- appropriately representative.

MS. TERRY: So, yeah, so we've thought a lot about this, certainly before PCORnet and during, and serving on the executive committee of

PCORnet I've begun to think about it more globally. So, we are -- built into my project are navigators and I know several other PPRN are doing a similar thing, so these are individuals who are trained to do phone calls, to do visits, that sort of thing. That's really highly expensive in the sense of person power.

We have the opportunity here though to long tail that and get lots and lots of people to do it because these armies of people are very interested in furthering research more quickly. So, I think that's one way.

We've also looked at a whole host of things. I just came -- this morning I spoke with the ONC Consumer Health thing and so did Jean, and there the people representing underserved and marginalized communities talked a lot about, we are not non-digital, because that's one of the push backs we get a lot is, this is all digital and digital natives love it but other people don't. So, text-based systems, which are harder to create and more expensive, which is crazy, are one thing

that we're looking at. Using the infrastructure that already exists in communities -- so, church-based and community-based organizations -- and there's a whole body of work that's already been done by like Community Campus Partnerships, National Council of La Raza, et cetera, that I think we have not tapped enough, and I know I haven't enough. I do in other parts of Genetic Alliance, but not this, because I don't have enough time.

So, I think resources in terms of time to be able to integrate those things and PCORI -- PCORnet integrating those things on a PCORnet level would be really good.

And then as far as, you know, for example, the nine groups and what do they want to do, I'm kind of amazed -- again, the advocacy community, especially around disease-specific stuff, is very old. We are 50 years old, some of us. My group is only 20, but there's others that are up there, and I think the newer forms of being in affinity with one another, like Facebook, et cetera, are

overwhelming the brick and mortar groups and they don't quite know what they want to do. So, they know they want to accelerate research and they know they want to provide services for their community and listen to them, and vis-à-vis the example Holly gave, we have lots of examples like that, and I think what they've finally begun to understand is that they are able to do this if they, one, do take care of those communities, so they keep in connection whatever way they need to, and two, if they start to look at cross-condition issues.

So, what we've found when we brought the nine groups together is, we took nine plain old patients from those groups, not the leadership, and those people said, we deal with fatigue, we deal with the burden of illness, we deal with familial and marital issues because of the stress of disease, we deal with pain, we deal with -- and they just went on and on wanting to answer those questions together and not be just identified with hepatitis or with inflammatory breast cancer.

So, I think what we're finding is there's

a kind of -- a people-ness, just like I think people are not patients, I think people also don't identify just with their disease, and we need to figure out how to connect them better and I think we can do that in something like PCORnet.

MS. PEAY: So, I won't pretend to have an answer to this question but I'll tell you some of the ways that we're trying. This is really hard.

So, in a rare disease community there are special ways, especially with a disease like ours, so most individuals with Duchenne or Becker muscular dystrophy are seen at least once a year in a specialty clinic. So, our obvious focus, and what we've really been pushing, is clinics -- clinicians and clinics themselves, and there's a lot of politics involved with that, especially in our world where there's a lot of organizational politics and then clinician and advocacy politics.

But that is the obvious thing because we know that the large majority go there at least once a year.

The second is that we have grassroots

groups, so it's a similar thing, like you're talking about, with a community involvement and getting the word out in our community and we've been really pushing that. But we still find that even in those groups that we still get a select group of people who stay engaged. They might start engaged, but they don't stay engaged.

So, some of it, again, like Sharon said, is trying to make sure that we're doing something that's interesting, and this is an opt-in situation where people come to us and our history has been clinical trials and clinical trial readiness, and there's a large number of people who are just not interested in that.

So, we are working very hard on sort of rebranding ourselves and reorienting ourselves towards things that matter to a different subset of the population, and so making sure that we do talk about things like, you know, we're trying to gather more information about the pain that your children have and then as an adult you experience, and making sure that we are broadening our scope so

that it's not just perceived as a network that's ready for clinical trials.

But I don't know if we're going to be successful, and maybe at the end of 12 months I'll have a better answer of how well we've done.

CHAIRMAN NORQUIST: Thanks. One thing I would say, because working with a lot of underserved populations that one of the ways to engage is to offer something else to people who are struggling every day in life, and so to go to them and say, well, I want you to participate in this, that's one thing, but then to offer something else.

So, for example, there's this woman who does a lot of cancer outreach up in the Mississippi Delta, Romana actually came with me, so one of the things they do is actually offer a screening and then get them to appointments and things like that, and then they engage them in this. So, that's another option of how you get people who see some benefit other than the overall benefit.

DR. HESS: Yeah, so we offer free genetic testing and it hasn't helped yet, but I'll let you

know.

CHAIRMAN NORQUIST: Yeah, genetic is different than come see if you have a tumor in your throat or something. So, I agree. We'll see. Rick Kronick is sitting there.

MR. KRONICK: Thank you. Thank you all. Really fascinating and incredible work in a short period of time. My question is, I think, probably mostly for Rachel, and it's -- as a background, we are dealing in the government now with requirements for open data. Everything we fund, you know, researchers need to make the data available at the end of the work, and, you know, PCORI will be putting in, if this is approved, maybe something like $250 million all together into PCORnet and I wonder -- and I'm struck by Holly's comment about, you know, selling the data and clearly the sustainability question is front and center, which conflicts with open data.

But I wonder what your thoughts are about the availability of this at the end.

MS. FLEURENCE: So, well, I think several

things. We're still in the early stages of developing the governance policies for PCORnet, but one -- so, one thing that's clear, though, is that PCORnet research studies will be subject to the same rules that apply to PCORI research. So, any rules that we have around access to data that PCORI will be supporting will also have to apply to PCORnet.

The other thing I'd say --

MR. KRONICK: I'm not familiar with those rules.

MS. FLEURENCE: So --

DR. SELBY: Well, for example, the rules we just mentioned this morning, the proposed rules for peer review and release of research findings and then the open science approach that Harlan and Steve Goodman have been leading and we talked about last week on the call.

So, we'll be coming back to, you know, a more refined approach to that, and as Rachael said, it will influence any research that PCORI funds in PCORnet. I don't think you could say that it

applies to the infrastructure data that the networks hold. In fact, a lot of that data, correct me if I'm wrong, you guys, but I think some of it will even still sit primarily in the partners within your network and it will be brought forward for particular studies.

MS. FLEURENCE: So, I think it will be important to differentiate between sort of the data being housed in the virtual data warehouses within each CDRN, which will not be open, with case-by-case research, research studies that will be done, and then they'll be subject to a certain amount of rules that will be done through the governance and will most likely include open data and sort of the whole open science policy that PCORI is pursuing.

The other thing about selling data, and I'll let Holly correct me, is that my assumption would be that since you have patient governance on your network, the selling of the data is not done sort of without the knowledge and the buy-in of our patients. So, I think that's a really key distinction is that this is not data that's being

sold without the knowledge of the patient.

MS. PEAY: So, can I just clarify though? Because when I say sell data, I probably did use that term but it was not exactly accurate.

So, industry wants prep to research data, they want very specific counts of people in particular areas with particular phenotypes, and so we do that kind of cutting of the data. This would not preclude us using end data for any other reason, and then the way that we actually predominantly work with industry is recruitment. So, it's not actually giving them any data, it's actually using our data to be recruitment.

So, I apologize if I was unclear about that.

MS. TERRY: And I would add to that, I'm a huge open everything proponent, as some people around this table know, and what we're looking at is the service of serving up that data in a way that makes sure that it's quality controlled, et cetera, being the thing that we sell, which is very similar to what DuchenneConnect does, not selling

the data itself. So, they're not incompatible unless the government decided it was in charge of all data and in charge of the service the way they are, for example, with PubMed Central.

CHAIRMAN NORQUIST: Harlan Weisman.

DR. WEISMAN: I have two questions, one specifically, I think, for Rachael and it relates to page 142, but it's on the CDRN requirements. One of the things that I -- that's been important to me in this whole PCORI process building this PCORnet to begin with specifically is broadening our definition of who investigators are to include patients and other stakeholders as not people who just go to meetings and say what's important to them, they actually participate in the research. Yet on the bullet it said, you specifically pick out members who are already in the club of investigators.

And so I was just interested in that, that you -- I mean, clearly, it goes without saying that everything we do, the highest standards of scientific rigor and quality that we've talked

about all day, but given that, I'm just -- I think it's important to broaden as opposed to make sure that we narrowly focus, and I was interested in why you chose that.

MS. FLEURENCE: I'm not sure that --

DR. WEISMAN: To break it out as opposed --

MS. FLEURENCE: I'm not sure which bullet you're referring to.

DR. WEISMAN: It's the one about having clinical researchers as a principle investigator or a co-PI. I have nothing against that but you also spoke perhaps, I think, about having a patient or some other stakeholder as a PI or co-PI.

MS. FLEURENCE: That's a good point. I think we were really responding to sort of the increasing realization that in Phase II this really has to be about research and improving the efficiency of conducting research, and I think sort of as we pivot from infrastructure building to actually conducting clinical research, we want to make sure our CDRNs are staffed appropriately to be able to sort of have that big picture understanding

of what it requires to be able to conduct high quality research. So, that's really sort of the impetus behind this.

I think it in no way precludes having a co-investigator who may be a patient or another kind of stakeholder. By Phase II, however, there will be patients and stakeholders involved in the governance and on the leadership of those CDRNs and PPRNs, they wouldn't get funded for Phase II if they did not have that well in place.

I think the particular point about just saying make sure you have a clinical researcher as a PI or co-PI is just sort of really making sure that we set them up to be successful to pivot from infrastructure to research.

DR. WEISMAN: Right, and my point is I -- you know, we spent a lot of time talking about the investigator and how they should publish, and we don't want to get in their way, but I don't see any reason why a patient couldn't be at a podium presenting research and there are a lot of academic researchers whose quality and rigor leaves a lot to

be desired. So, to me, the basic principle is to make sure that there are people who know what they're doing and have been well trained and have a reasonable track record. Okay, enough of that.

The second question, I guess, is for the broader group and maybe for PCORI staff, which is, now, when I was listening to this, we have -- each of the -- well, many of the CDRNs and PPRNs are a network of networks and then we have PCORnet, which is the mother of networks, I guess, I mean that in a nice way, and that allows us to reach in and do a lot of research, but I was curious about -- and it allows people to aggregate interests together when it makes sense and break apart and not work together when it doesn't make sense, and I love that.

What about the process by which knowledge and learning and I think, Sharon, maybe you used the term learning healthcare, when you did that system, how do we make sure that there's good distribution of what people are learning both informally in setting up networks and how they work

and what works in engagement and what doesn't and so forth, but also in research findings taking something that was studied, for example, in a randomized clinical trial, and looking at its applicability more broadly and generally through testing within the network of network? That connectedness of how people communicate, I guess.

MS. FLEURENCE: So, I think I'll answer in two parts and I'll start with sort of the research findings. I mean, I think again, the same answers I gave to Rick is that research studies funded within PCORnet will be subject to the same rules that they are within PCORI and sort of implementation and dissemination will be key criteria for funding these research projects.

So, the same bar will be there for the --

DR. WEISMAN: I'm thinking more in terms of a formally study invalidating that what we found here within this part of the network, we tested it subsequently, whether it actually is true in a different population or a broader or more diverse population that's available within the network.

MS. FLEURENCE: So, these are definitely areas that we can investigate or processes that we can investigate. I think we're not quite there yet. We're close to being there, but not quite there. The other answer I was going to give you --

DR. WEISMAN: It's really just learning from each other.

MS. FLEURENCE: The learning from each other, I mean, I think we all aspire to do that and we're all looking at ways to do that. We really are sprinting though; this is really an 18-month sprint.

So, right now we have sort of informal ways that folks have gathered together around interests and they're sharing informally, but we have our PCORnet steering committee, we have CDRN and PPRN PI retreats. We've not, I think, had time to stop to catch our breath to do something more formal in terms of dissemination learnings, but I think we're probably close to being able to do that, maybe in the winter of 2015, but this has been a real sprint to ramp up. I mean, these are

29 networks, so I have 25 other PIs like those that we invited today who are really working full out to do this.

CHAIRMAN NORQUIST: It's a spring; we got that. And then there's work to be done and there will be an opportunity to certainly try to interface here and have people working together and learning from each other. Okay. Let me -- Allen and Christine have the last and then Freda, if you want to say something at the end here. Allen?

DR. DOUMA: Allen Douma. Again, thank you very much. It's been delightful and exciting to listen to all of what you're going through now and looking forward to what you're going to do in the future. With regard to sustainability, though, and this is directly to PCORI folks, what I hear is the sustainability for you guys in the long run is going to be based on your individual ability to market yourselves to people who want to get research done.

My idea, at least, when we first conceived PCORnet is that the power is in the numbers and

that the PCORnet itself needs to market itself, not have the individual members, and I'd suggest that given we want to have sustainability plans hopefully start the first day of the third year, not the last day of the third year, that we need to begin to start marketing PCORnet as a whole now and the beauty of doing that is we will find out from our potential customers what it is they need and want, which will help each individual member of PCORnet, the CDRNs, the PPRNs, to be able to actually develop a product, which is going to meet the needs two years from now.

CHAIRMAN NORQUIST: All right, Christine and then we've got Leah and Ellen and -- so, let's make these quick because we're running out of time.

MS. GOERTZ: Hi. Christine Goertz. I also want to thank all of you for the incredibly hard work -- incredible hard work that I know you're doing to make this dream really become a reality. It's easy for us to sit here and say, oh, let there be something like PCORnet, and you are the ones that are putting in the hundreds of hours

to make it happen. So, thank you very much, all of you, including Rachael.

My question is for Rachael. Can you give us some idea of what the benchmarks are for success for Phase I, both for individual -- are there sort of individual benchmarks for the PPRNs and CDRNs? And then also collectively, as we're looking at -- you know, are we -- is this working or --

MS. FLEURENCE: So, this could be a very, very long answer, so I'm looking at Gray.

CHAIRMAN NORQUIST: Can you -- we should be able -- we have that somewhere, but I mean you should be able to get to the point.

MS. FLEURENCE: I'll try. So, for individual networks, there are a number of milestones that they need to meet in terms of their governance, their patient engagement, setting priorities, including their patients and their clinicians, organizing their data correctly, streamlining their IRB, streamlining contracting, so a number of items that they, individually as networks, need to be able to achieve.

Having said that, we also want PCORnet to be successful as PCORnet, so we're not funding 29 individual networks.

They do have to collaborate together and I think the success of PCORnet as a whole is being able to leverage multicenter research, so being able to have large sample sizes, do the testing that Harlan was referring to about generalized ability, being able to have data organized in similar formats so that we can run large observational studies quickly, doing prep to research. So, there's a number, I think, of milestones around the data, around the governance of PCORnet itself, so how do you get studies quickly approved and run within PCORnet?

So, they're a little separate because we could have 29 successful individual networks and still not have a successful PCORnet, so they do need to be looked at together.

CHAIRMAN NORQUIST: So, we have parameters on which to measure. They know what -- you know what you're supposed to be doing, right, by the end

of this, so they know that and that's really key and that will be there for when we evaluate them to move on to the second phase, is that correct?

MS. FLEURENCE: That's correct.

CHAIRMAN NORQUIST: Okay, thank you. Leah.

MS. HOLE-MARSHALL: My comment was similar, and thank you again for all your hard work, but it was really related to quantifying the outcome or the measures in terms of whether we're getting to where we need to go, and with respect to the sprint, if we've set aggressive timeframe, that's okay if we're not accomplishing everything we thought we would do, but at some point we need to articulate what that is and when we would be able to get to a point where we can actually measure the outcome we want to have, which is accelerated research and some of these other networks.

So, I'm hoping that if we're moving to Phase II, we're at least thinking about when we will be able to assess that and what we will use to

assess it by, so even if we can't assess it at the end of Phase I, which I don't think we can do, I'm not saying that that's a failure, but we really need to be able to articulate that and I worry a little bit in this description of, you know, describe plans for how you're going to progress towards clinical research after another three years seems a little underwhelming in terms of where we might want to be.

So, I just want to put that out there --

MS. FLEURENCE: Well, I'm sorry if I gave that impression because actually we are -- we're going to be increasingly turning on the pressure to our networks to go in for joint applications of research as PCORnet even before Phase II kicks off. We have a first use case with our aspirin trial, which is -- the protocol's being written as we speak and the CDRNs are collaborating right now in order to put in a full application, so we'll get early signs of how that collaboration works. We have the obesity cohort, which we hope to be able to see some real research questions coming out of

that, not just sort of being able to organize the data around that.

So, I'd say we do have early signs and we'll be looking at these very closely as we get towards Phase II to see how successful the networks are in engaging around these questions.

MS. HOLE-MARSHALL: I think it was more of the descriptors that were up there.

CHAIRMAN NORQUIST: Yeah, and I think I heard also from them that they're also discussing with other potential partners too other than us about potential research, so that's set up already.

MS. HOLE-MARSHALL: The only other question that I had, and I don't need a response, but I am curious about the reasoning behind extending beyond our initial group to a larger group, and there must have been some success measures where we decided that the investment was successful and now we're going to extend it without knowing the full measure of success, which is where we want to be. So, I don't need a response at this point but I think it is --

CHAIRMAN NORQUIST: I don't think that's something that -- that's a legitimate issue is about why go for more when you know what we need -- what you've got now or whatever, so can you quickly potentially say why we are moving up to a larger number?

MS. FLEURENCE: Well, it's only slightly larger and it's "up to", so it doesn't necessarily mean we will fund up to that number. I'd say we're aware that there are networks out there that are very sort of high performing networks and they would have to come in at sort of a high level already, so they wouldn't have a long ramp up period, they would have to be fairly functional fairly quickly.

But from the beginning, we talked about PCORnet as being open and it just -- I think it didn't feel right to sort of close it for Phase II to new applicants, new entrants.

MS. HOLE-MARSHALL: Well, you could do that, though, by keeping it at the same number but making sure that everyone that currently has a

grant is high performing and competing against others that are high performing. It's really expanding the program that I'm talking about. You don't have that evidence yet that it's doing what we want it to do.

CHAIRMAN NORQUIST: Yes. That, I think, is for more discussion because partly we need to know what we're going to use the PCORnet for, for the ideas about what it is that we want to do, and that may come in with the review, actually, when the review comes up, we may have less than 22, we may end up with 15, but it's a very good point and I think something to keep aware of. Yes. Ellen, are you still going to ask a question or you put your card --

MS. SIGAL: It's not a question, just a statement. I want to thank you for your work, I think it's incredibly important and I love the fact that you said that the doctor doesn't know everything. I recently had to speak at -- on diagnostics at Mass General and I made a similar statement that, you know -- and the doctors on the

panel were really angry. What are you talking about patient's needs, trust their doctors? This isn't about trusting, but I just felt compelled to say that. I'm sorry.

CHAIRMAN NORQUIST: Let me just remind you, as a physician, but I would just say that there are the clinicians, too, other than physicians, who don't know what they're talking about also. I don't want to let them off the hook.

[Simultaneous discussion.]

CHAIRMAN NORQUIST: That's what I want to do. Let's offend everyone instead of just the physicians.

MS. SIGAL: [Off microphone] and not knowing what you're talking about.

CHAIRMAN NORQUIST: Well, that's true. None of us -- nobody knows everything, right.

DR. DOUMA: I solve that problem by being mute. Something to consider a model going forward, particularly a couple years from now, maybe somebody wants to join the PCOR network who we haven't funded at all, because being a part of the

network is such an advantage, and so we ought to put the word out for later on.

CHAIRMAN NORQUIST: Well, we hope that's the case. Right? So, Freda, since you've been overseeing this with your strategy committee, we want to give you the last word.

DR. LEWIS-HALL: Yeah, actually, though, I wanted to do a lightening round because the questions that are being asked right now are the questions that, as a committee, we keep going back and forth on and that is, is the next -- expanding the network to begin to get alignment and more capture or is it creating greater depth in being able to complete research that we anticipate we could do? And we know what we think and I just wanted to do a lightning round across the four of you --

CHAIRMAN NORQUIST: You want to ask them? Yes. So, you know, you brought up a good point, though, that I want to be clear about is that many of these questions, I think Leah and these others, that are very important, come back to your strategy

committee to really -- because we're not -- this is not sealed, this is what we're -- you know, this is, is this where we want to be, but this is -- opens up more discussion.

But I think it would be interesting to hear certainly what your feelings are. So, we'll just go from left to right.

MS. TERRY: Sure, so I think deeper and I think on the PCORnet level, and that means engaging us because actually we've begun to think about, do we have the right metrics? We've had a retreat for the executive committee on creating metrics that we think are going to change the world, because we're giving our skin to this thing. So, I think asking PCORnet and also the 29 or however many funded entities to start to say, how does the world change if PCORnet is functional, including funded and un-funded entities? How are we not just CTSAs or collaboratory or whatever else? How are we going to change the world?

MS. PEAY: So, I agree with that. I think that's very helpful and I think one of the things

that we struggle with as a rare disease group, and I know more rare disease groups would be dying to get into this network, but the existing PCORnet activities really don't address issues with -- no one's going to want my guys in the ASPEN trial, no one's going to want my guys in the cardiac trial because they have so many comorbidities and it's predominantly men, that's why I'm saying guys.

You know, I think there's some real questions about how especially rare disease PPRNs fit in this big picture and I think answering those questions really fast would be very helpful to making that decision.

CHAIRMAN NORQUIST: Thank you. I'm glad you pointed that out because the rare disease issue is always a special kind of issue sometimes that we need to focus on. Thank you.

DR. HESS: I think that there's a depth and a breadth issue. I think that the funding level that's being talked about for Phase II is probably close to appropriate to continue the activities and to dive more completely into those

activities with the idea that we're sustaining infrastructure, not sustaining research.

I think that PCORnet and the CTSA, NCATS, and the other initiatives that are being developed have created a space in which academic medical centers either participate in them or fold and I don't think that that's hyperbole. I think that that's a reality and to think about -- there's a lot of breadth in the inclusion of PCORnet right now and of NCATS and their initiative, but it's not complete and thinking about the message that's sent in terms of the value of some institutions that maybe could not have gotten it together quickly enough for the Phase I of PCORnet is something that one needs to think about very, very carefully in making those decisions.

CHAIRMAN NORQUIST: Thank you. Dr. Carton?

MR. CARTON: Two quick points, one is I think that there's a balance between the depth and the breadth. There's definitely a need for depth. I think these numbers are balanced. I feel like

other networks that have different experiences from PCORnet joining at the second phase could add to the initial investment in terms of the collaboration and the learning process.

To the other point about joining PCORnet willingly, we deal with that all the time. I mean, we, as a central node, funded our partners to participate and we're constantly trying to develop strategies where we could go to other systems within our state and ask them to join the LACDRN, obviously at smaller levels than we started with, but then develop the benefit case that they would want to join on their own and won't really necessarily need to be seed funded like we did in Phase I.

CHAIRMAN NORQUIST: Thank you. Freda, just --

DR. LEWIS-HALL: Yeah, I just wanted to -- I jumped into the question but I would be remiss to not say thank you all for coming here today and sharing your thoughts, your incredible work, and then to the staff of course, and the CDRNs and

PPRNs who are not here, and to the RTC, because we jumped into the deep end of the pool quite quickly and I want to thank the members.

CHAIRMAN NORQUIST: Yeah, so let me add to that and just -- on behalf of the whole Board, those who didn't speak, and I'll speak for them and add also thank you very much for this, and also for those who -- I'm glad you said that, for those who are not here, we have four out of the great number, and for the work that your committee and also the SOC and others have done as an input in on this also, and to be continued, right? Thank you all very much.

[Applause.]

CHAIRMAN NORQUIST: Robin Newhouse, you're up.

So, we will have a break, we will adjust accordingly, but we do have an important discussion after that about our health systems program and then we do have the public comment period, which we do need to have also. We're alerting people on the phone. We will have a break after this, so Robin

is up against the break.

MS. NEWHOUSE: What we will do is update you on some of the activities of the Methodology Committee and we will talk about five things, first of all, an update on the Methodology Standards, the Dissemination and Implementation Plan, second, to update you on the development of new standards, then talk about the methods monitoring in the portfolio, a couple specific activities, an update on our discussion about the translation framework and what will come next, the Network Research Methods Workgroup, and then we have a couple more updates.

And I've asked David to join me particularly for this first part of the presentation around the dissemination and implementation update, because truly the work that's been done over the past year has been a major effort on behalf of the PCORI staff and David and Katie have led some of that effort within PCORI.

So, first item is dissemination and

implementation of the Methodology Standards, and the first audience was the internal audience. It was important for PCORI first to build capacity within PCORI to implement the Methodology Standards and be able to support those applicants that were going to use the Methodology Standards.

So, the intended outcomes for the dissemination and implementation plan is here, first, to increase research in stakeholder awareness of the standards, to incorporate the standards into key PCORI programs and activities, increase the standards use by researchers, publishers, and funders, and increase the availability of tools and other resources to facilitate the use of standards.

So, the implementation plan was reviewed by the Board at the end of last year, so the beginning of this fiscal year and the first activity was to operationalize the standards in merit review process.

So, at this point, the Methodology Standards have been incorporated into the guidance

for the PFAs, the staff have launched that internal workgroup that I mentioned led by David and Katie.

They have developed some resources for evaluating the standard's use by the applicants, and of course the Methodology Committee has gotten a report of the use of the standards for applicants that have submitted proposals to PCORI. And the next step for the staff is to develop adherence and an interpretation guide that will be available this fall.

The second set of activities has been around dissemination. There have been a number of webinars and presentations, this first one there were 500 registrations for a July webinar that was held, and the staff are tracking the journal and scholarly work, and as one might expect, many of the citations relate to the Methodology Standards started around the publications that were done by PCORI staff and PCORI related people, and now we're starting to see more publications outside of the PCORI network being cited.

So, next step is more publications in

terms of Methodology Standards to get that out in the peer review literature and also additional partnerships externally.

Standard training activities have been prevalent. The PCORI staff have undergone or they've completed a three-part training session on the Methodology Standards. There has been extensive merit review training as well as a survey of the merit reviewers to determine whether the training has been sufficient and their experience with the merit review in terms of the Methodology Standards.

The next step for the following year is to modify the existing training materials for the external audience. The next year we'll also include a number of monitoring and evaluation activities, including performance metrics for implementation to understand the reach that the Methodology Standards have had, that will be coordinated by the Standards Workgroup with input from the Methodology Committee, and then development of decision support tools for

researchers and reviewers.

So, David, would you like to add anything to this?

DR. HICKAM: So, I think that one of the significant pieces of this is that, you know, the Methodology Standards were developed by the Methodology Committee, you know, at a time when PCORI staff was still pretty small and the whole process for conducting merit review and managing portfolio projects was just getting started.

And I think one of the important things that's happened in the last year is that the Methodology Standards have sort of been brought in and sort of brought into the culture of PCORI so that we felt like that it was useful to sort of have the first initiative of training materials and really just practical experience using the Methodology Standards to be done internally and that that's actually gone extremely well.

I think that if you talk to the staff, I think they feel like they've got a much better appreciation about what the standards are, which

are a set of guidelines for helping to ensure best practices in clinical research.

And we feel that this is a sort of a good sort of platform for launching additional training materials to outside audiences, which is just sort of getting underway now.

The other thing is that I think, you know, the one thing that you gain an appreciation for sort of working with the standards and applying them to individual projects is to get, really, sort of a good comfort level that these really provide a vocabulary for discussing methodological issues with the people that we make awards to. And so, as I see the process of adherence to the standards is really an ongoing activity.

It's a way to share information about the guidance with the awardees and sort of work with them on an ongoing basis to sort of improve their projects and refine the methodology, and so, you know, I think that we sort of realized that, you know, trying to measure or develop measures or metrics that would show how this was being used,

it's not just a percentage of this or a percentage of that, but it's really to sort of be able to sort of capture how it helps inform the relationship between PCORI and the awardees and really to get the perspectives of the awardees about how helpful that process has been.

So, Robin, that was probably more than you wanted to hear from me, but hopefully it gives you sort of a sense about how we're trying to operationalize these into our everyday activities.

MS. NEWHOUSE: Thank you, David.

The next update is in terms of the two standards that we're currently working on, the first is the design with clusters and the idea in the development of this standard is a little different than we used to our approach for developing the standards in the last contract period.

In this situation there are a number of people that have worked in the area of designs with clusters, we've made multiple contacts with those experts and we'll be planning a workshop, actually,

this fall and we have a couple dates secured, so I'm not able to tell you that, but the idea will be we've drafted a set of standards based on the prior work for designs with clusters and that workshop will have refine and come to consensus on the development of those standards.

The second set of standards is related to complex interventions, and we actually have a meeting on Wednesday in which we will come to some conclusion about the definition of complex interventions. We, last year, reviewed a number of definitions. There are also people that are working in this space in development of guidelines for complex interventions. Many of the studies that PCORI funds, particularly in health systems, which is care coordination, are, in fact, complex, and we have established a draft approach to the development of these standards. Also, this will be a little bit different than we had done in the past in terms of the contract, but we have a meeting on Wednesday where we'll flesh out our approach to standards for complex interventions.

The next item is related to methods monitoring in the portfolio, and one of the first reports that PCORI staff has provided for the Methodology Committee relate to the types of project that the PCORI methods research portfolio has funded, and we were able to understand at least a description of the 41 studies that are funded in the methods portfolio and how they're spread across some of the current methods standards and have begun to discuss where some of the gaps are, that will be another point of discussion about where we need to focus in terms of growing the methods portfolio.

The second is in terms of the Clinical Trial Advisory Panel. Of course, CTAP has been endorsed and is now going strong. Steve Goodman and Mary Tinetti have joined the Advisory Panel as ad hoc and they have invited us -- they've developed three priority areas that they'd like to start working and that's recruitment and retention, monitoring clinical trials, and some common definitions, have invited Methodology Committee

members to become part of that subgroup so we're often involved, all of us, with CTAP subcommittees as well.

In addition, the third item related to monitoring in the portfolio, in terms of implementation of the standards, we mentioned that the PCORI staff was already evaluating the adoption of the Methodology Standards within the proposal and has provided a report to the Methodology Committee, but we had also discussed the option of a methodological consultation and that will be implemented in the first round of the pragmatic clinical studies in November of 2014.

This initiative is being operationalized by the staff. The idea is that there would be some methodological expertise that would provide a consultation to the review of the proposals to strengthen the rigor of the proposals and make some recommendations if, in fact, there are recommendations to be made in terms of ways to strengthen those proposals.

The next item I wanted to mention is

related to the translation table, and you see from the statute that the translation table was really provided to provide you with guidance and to act as a reference to you around research methods that were most likely to address each specific research question posed.

So, to date, the Methodology Committee proposed a translation framework, and I'll just go ahead here, just to refresh your memory, that provided a logical flow in terms of generating a question, evaluating the evidence that's been synthesized around that question, framing it in a PICOT framework, making sure that it was a priority for stakeholders and patients, and then identified a number of intrinsic characteristics and extrinsic characteristics by which you weighed the importance to make a determination of the design and methods that are used and the study selection.

So, the Methodology Committee had worked on a number of approaches to a table and -- a two by two table or a flat table was not something that was incredibly helpful. We evaluated the use of

some algorithms and came to consensus on an approach that used these intrinsic and extrinsic factors to help weigh the importance of each design and method.

So, under discussion at the meeting on Wednesday will be an approach to a next step to come to consensus on the best way to be helpful in terms of helping the Board on recommending design and methods.

Another initiative that the Methodology Committee has been involved in, and this was another initiative that was intended to help PCORI in terms of methodological guidance was in the PCORnet and there were two areas that the Methodology Committee supported to move forward on to provide some methodological expertise to PCORnet and that was in terms of distributed analysis and the second being on the topic of missing data that I'll mention.

So, to date, Sebastian has led a group to evaluate these methods and distributive analysis and there will be a workshop in the fall --

actually, I think it's December, but a date will be forthcoming, to work with experts in terms of identifying those issues, those methods issues, and there will be a dynamic relationship between this PCORnet methods group and the Methodology Committee, not only to provide expertise for the Methodology Committee, but also to identify areas where we might be able to develop additional standards that might be helpful.

The second area was led by Sally Morton and, as I mentioned, handling missing data and inconsistent data, and that group is a little -- not as far ahead as Sebastian's group and there is an expert committee forming to refine their scope of work.

Other updates related to the Methodology Committee, there is also a development of a plan to host a workshop in decision science. That will be held in November of 2014. The second issue, the Value of Information RFI announcement was released in July to collect input from stakeholders externally and a public webinar was held in August

with final input due related to Value of Information September 5, 2014.

And then the last update, being we are awaiting announcement from the GAO regarding appointment of new Methodology Committee members. We had four openings that were related to Sherine Gabriel, Sharon-Lise Normand, John Ioannidis, and Al Berg, and so we miss them terribly. So, we are waiting patiently for the next group of Methodology Committee members that will be appointed by the GAO.

So, let me stop there and, David, any additional points you'd like to make?

DR. HICKAM: I think that was an excellent summary and really shows all the multiple sort of parallel lines of activity that the Methodology Committee has been pursuing this year.

VICE CHAIRMAN LIPSTEIN [Chairing]: So, this is Steve Lipstein. I'm substituting for Dr. Norquist, who stepped out of the room very briefly. Let me see if there are any questions. Harlan?

DR. KRUMHOLZ: Thanks very much. I have

three quick comments, one is on the Value of Information. Are you going to be -- the Methodology Committee going to be applying the best methods of Value of Information to our own portfolio in informing us about what your thoughts are based on the criteria that you developed?

MS. NEWHOUSE: So, I would say that if that's something you'd like the Methodology Committee to do, that that would be something that we would be interested -- we've had a number of conversations about how VOI would be used and actually if you would like us to generate criteria, we absolutely will.

DR. KRUMHOLZ: I'm just throwing this out just for -- in the ether for us to think about. If we're going to invest in a Value of Information announcement, if we're saying this is the kind of way that people should be thinking about research, at some point we should submit ourselves to this kind of evaluation. I don't know when it should occur, but I'm just thinking if it's deemed, based on this investment, that this is a good approach,

then we ought to be willing to submit ourselves and actually probably ask our colleagues at AHRQ and NIH whether they'd be test use cases for some of this in some circumscribed areas to try to figure out how we're going to get the common parlance, how we would bring this in and evaluate ours and theirs. Just as a quick point.

The second thing is I also think you guys have got a lot to do, but in terms of prioritization, it would be interesting to ask whether or not the Methodology Committee could give us some guidance about how we would peer review content articles or results of grants against the criteria that they've developed. What kind of tools do we need?

Again, when I say this, it doesn't mean that you guys should actually be doing the day-to-day work, but that you might commission somebody to do work under your guidance and that we would probably benefit from your perspective on how do you do this, because you've now set the criteria. How would we evaluate whether someone's complied

and what are the calipers that we use? I mean, if they're within plus or minus -- if they're close, is that good enough or are we going to be really exact that they've got to follow the letter of the guidance? And to what extent -- how is it transformed from a guidance document to one that's actually an accountability document? And that's where we deal with all this time in performance measurement. It's one thing to put out guidelines, it's a whole other thing to hold people accountable.

And I think what we've got is a situation where we've got great guidance. How are we going to use that for accountability? It seems like we're mandated to. And it would be really good to get the Methodology Committee's input into how that transition occurs.

MS. NEWHOUSE: So, Harlan, when you talk about accountability, are you talking about the standards or are you talking about peer review process?

DR. KRUMHOLZ: The one that we talked

about earlier today that we're going to need to undertake, that we were legislatively mandated to do prior to -- as part of the reporting piece, and just saying funneling in the wisdom of the Methodology Committee and the larger community, crowd sourcing further, but how you guys can catalyze that might be very useful.

And then my third, and I know you've heard this and I'm just saying it for the Board, I thought that at this time we would already have materials that we'd be disseminating that teachers across the country could be integrating into their course work and at various different levels if people wanted to learn about PCOR and CER. I thought the Board endorsed that in the strongest possible terms that we thought this was critical for us to accelerate the dissemination of the work of the Methodology Committee both to honor the work that they had done and in recognition of its depth and strength, and I want to know what we can do -- so, at this point we don't have that yet. I know we have a contractor, but it's not clear to me

what's being put out.

So, I'm just saying on an accountability basis, when would we expect to be able to distribute not to people like all of us might want to teach and that we would benefit from those materials, but for people who aren't in the midst of it but would like to be able to integrate it in every school of public health and in every medical school and in every nursing school and so forth throughout the country, where -- how do we take what you've done and now turn that into it? And, like I said, I thought that we had expressed that, but maybe we hadn't expressed it clear enough because it seems to me like now's a good time.

DR. HICKAM: Yes, we're very aware of that request and we're looking to move those kinds of materials out to the general public this fall. I think that was on one of the slides.

DR. KRUMHOLZ: But what exactly are you moving out and when?

DR. HICKAM: Well, the model is a couple of things, one is sort of additional text matter

that really provides sort of further sort of contextual interpretation of the standards and then the companion pieces are slide sets and sort of curriculum notes or guidelines that could help people to know how to use those materials. It's kind of a user's guide idea, but in terms of what would people actually use in a course, you know, we're sort of reviewing that the slide sets would be one important piece of that and we certainly would be interested in other suggestions that you might make.

DR. KRUMHOLZ: Well, let me just reiterate what I thought I had suggested at the prior meeting, which is that these might be good and necessary, but I don't think they're sufficient.

And, you know, it's hard work to develop a curriculum and my thought was that we would commission somebody who would help us create it based on the work that had been done and in concert with the Methodology Committee education goals to say, here are three potential curriculums that can be taught next year at the school of public health.

Now, each person, of course, at that point would tailor some of it, but I think a lot of people don't know quite -- these documents are daunting.

I mean, I've looked at them, they're like gold, but you've got to dig because they're not completely accessible to people who aren't in the field. You know, you guys did a great job to the extent that you could, but I mean, I think the translation for the use by teachers tools, not just slide sets, but actually setting goals and having material expectations, what might potential curricula look like, how might you cover it, people would mix and match that as they want to do it.

And I neglected to suggest every chiropractor school in the country as well, that's an important place to go, but every healthcare professional area, I think, would benefit from more knowledge about PCOR and CER. We're only going to make progress if we can go to our schools and say, hey, here's a ready-to-go curriculum that's built on the very best science and that's going to evolve

over time.

But I'm just saying, this is about the translation. Larry, you're always bringing up translation. How easy are we making it for people to pick this up? And I really think we've got to work hard and I don't think it can go beyond the backs of staff. I mean, we've got to be able -- you know, you've got to orchestrate this, but we've got to bring in people who can provide and develop these products.

Right, Harlan Weisman? I see you. I know you're engaged about this. This is one of your issues.

[Off microphone discussion.]

CHAIRMAN NORQUIST: So you're passing it back to me now. Okay. So, clearly, it hasn't been done. There's no reason -- wait, wait.

[Off microphone discussion.]

CHAIRMAN NORQUIST: So, clearly, it hasn't been done, it needs to be done, there's no reason that we can't contract this out. I mean, it's an easy -- I mean, there are plenty of people who

develop these kind of curricula and stuff like that that we could hand it to under the guidance, obviously, of the Methodology Committee. So, how long does that take? You could put this to somebody now who could do this within a month.

DR. KRUMHOLZ: And I want to say, I know that you haven't been standing still and it's hard to be sitting up there, so this is -- I want to acknowledge that staff has been working hard. I'm just trying to -- I think our job is strategy and prioritization and I'm just trying to say, on the Board -- and I'm curious to what my other Board members think -- it's not that you guys haven't been implementing, but I just want to say, this particular piece, I think, is a means to spread if my other Board members --

CHAIRMAN NORQUIST: Let me say that I did have a conversation with Robin about this and asking the Methodology Committee -- which I think on Wednesday when you meet you're going to go back through the implementation strategies and prioritize those, because that also had not been

quite honestly done. The staff has been very busy. We understand that. People miss the priority, but to me, this is an easy one that you don't even have to -- you know, you don't have to say, well, we don't have that -- we could easily contract this out on some of them.

Now, Sharon is waving furiously over here.

DR. LEVINE: So, I think someone said earlier today, we can do it fast or we can do it right, and I want to quote one of our panelists who said, nothing about us, without us, and I don't think you can do this in isolation from teachers who actually have to teach it. So, I don't think it's as simple as getting some educational consultant to be handed some material and craft a set of slides or whatever.

CHAIRMAN NORQUIST: Well, I think it depends on who -- I mean, I've seen this done before. It depends on the group to whom you go. If you just to go to some, yeah, right, I mean, some of these groups have teachers that are involved with them.

DR. LEVINE: Well, I'm saying that involving people who are going to be teaching this work in embedding curriculum design before saying here it is as a finished product.

CHAIRMAN NORQUIST: I guess my point is, I'm trying to take the burden off the staff right now and try to -- because we have funds that we could do this -- the appropriate group to do it correctly, I think, because it is something that we agreed we would do and it's clear the staff have a lot going on and it might be a good idea to think about who would be best for this with all the caveats and stuff so that we could get it done, but get it done expeditiously.

Because, I agree, I mean, I think we want to get these Methodology Standards out as broadly possible as we can to be used in stuff because that's a part of what we're supposed to do is change the way research is done, right?

So, I think that's a good point. Okay. So, Joe, you and I talked about this, but we should be able to --

DR. SELBY: Yeah, I would just ask you to ask us the same question in November. We will have a much more fleshed out --

[Simultaneous discussion.]

DR. SELBY: We couldn't agree more with Harlan.

CHAIRMAN NORQUIST: Okay. Steve, I don't remember what order we were going. We'll go with Allen.

DR. DOUMA: Great. Yeah, I sympathize with Harlan. I've been part of a communication with him about this and I think it's important, but I'm more broadly concerned about the slow pace.

The guidelines were available a couple years ago and one of the reasons that we're going slowly, and it's bit by bit, and we can answer this question, but there are another ten other questions that need to be incorporated into formal communication plans and by formal I mean that we've identified all the audiences, what we want each individual audience to do as a result of our communication, and then determine what's the best

way to deliver the information motivation for people to make those changes and have a formalized list of those things, each one of which takes a development process. You can't do them sequentially. You'll always be catching up. And that's the beauty of communication plans; it identifies everything at once. And so I would hope -- by November, it would be great -- if we actually had a formalized communications plan that we can then reference and put timelines in and know what the expectations ought to be.

CHAIRMAN NORQUIST: All right. Thanks. We've blown through our break, which we will need to take. We have five and we do have a very important topic after this, which is about our health systems portfolio too, which we want to cover too.

So, we can hopefully move quickly through these. Joe, are you still up for saying something or you just have your -- all right, now we're down to four. Leah?

MS. HOLE-MARSHALL: Thank you for the

presentation. It's great and I'm just so very pleased with our progress on implementing Methodology Standards.

The ones that we have now, as I continue to understand them, are basic or minimal standards. So, as we think about peer review, and I would really appreciate the Methodology Committee's perspective on this and hope that as you're prioritizing, we can think a little bit about -- to help us think about it, I hadn't thought of our peer review process as yet another check on whether researchers are adhering to basic standards that as a community we've all agreed are basic standards.

And so, while I agree that it's in the statute and needs to be addressed in some way, I'm hoping that we do that at the contract initiation or monitoring phase, and if we don't have an easy way to do that now, I'm hoping that you all will agree that it's important and help us figure out a way to do that, because I don't think we should wait until peer review to find out that the standards weren't followed and especially given

that they're minimal standards. So, that's my plea in terms of prioritization.

And, again, not that it's all on your shoulders, but I would really appreciate the feedback from the experts about how we might do that and do that efficiently.

MS. NEWHOUSE: Yeah, I would say we have good news. We just caucused and I'm going to let David -- and if he doesn't say everything I was thinking, I'll chip in.

DR. HICKAM: Well, I think we really have made a lot of progress over the last six months and we really now do have a well-trained staff that does do the review of the standards up front before the contracts are initiated.

I also would sort of repeat myself of what I said earlier. That review does identify issues that need to be followed up on and I think the other good piece of that is that we have sort of a system for following up on an ongoing basis on the issues that were identified.

CHAIRMAN NORQUIST: Thanks. Arnie? Oh.

MS. NEWHOUSE: I just want to mention the time that it took for the time that it took for the PCORI staff actually to develop tools to evaluate the proposals, to test them, to figure out that they had to prioritize with the focus on first, to make sure that it got everything that they needed to report back. So, that actually, I think, was part of the capacity building that took an amazing amount of time that was unexpected.

DR. GOODMAN: This is Steve. Can I just add something here?

CHAIRMAN NORQUIST: Sure, Steve.

DR. GOODMAN: I just want to mention that this is also exactly the function of the methodologic review or consultation process, which is to have methods experts, a team of them, look at these proposals at the submission stage after preliminary merit review and make sure that not only are basic standards met, but even higher standards that we would want for our research to meet.

So, that is precisely the goal and

function of that process.

CHAIRMAN NORQUIST: Thanks, Steve. Arnie?

DR. EPSTEIN: I want to speak in the context of the fact that I get to do something, which not everybody in the room does, I teach on health service methods and I found myself thinking that Harlan was really on to something when he thinks that that's a way to really inculcate and spread this, and I was thinking about what I'd like and it really comes off what Leah's comment is also.

The standards tend to be minimal standards, do this, do that, and it would be great to take that material and embed it in three to five lectures, two-hour sessions a piece, for PCOR of here's how you approach PCOR and think of the major ways we do it be it surveying, be it so forth, and then build in, as part of that context, is what a teacher would really like.

So, that's really just a really friendly amendment to say I think you're on to something wonderful. It's a great idea. This is not easy.

It won't be done by November and it will be -- you need somebody who's a teacher, you need, obviously, a methodologist, you probably --

CHAIRMAN NORQUIST: We might need someone in government. Yeah. They could be chief.

DR. EPSTEIN: I know I deserve that, but you don't have to give me everything I deserve.

MS. HOLE-MARSHALL: Also, free continuing medical education is a powerful tool. Free continuing medical education, if we could find someone to partner with us.

DR. KRUMHOLZ: There's no reason why we wouldn't ultimately want to do a Coursera or other online courses that would allow people around the world to pick up this stuff. I mean, once you start -- there's an appetite for this, plus, as we give out grants, people need to learn about it, plus, if we build it for patients and for health professionals at a wide variety of levels, then people can pick it up like that. I mean, the demand is going to be there.

The CTFAs are -- I think it's largely

related to what's going on in PCORI -- are requiring now patient engagement and PCOR to be part of the applications that are going to be coming in starting in December. There are large numbers of people who are smart people but are ignorant yet of the basic principles by which the Methodology Committee has laid down. And so we need easy ways for that to flow forward. The courses are one, but all sorts of things --

CHAIRMAN NORQUIST: Right. So, we're all very excited about education here, right? So, we will get to that. I wouldn't let him off the hook on November. If you don't keep a deadline, then it will go longer than that. Okay. I agree with you, it's probably going to take longer, but we're going to hold them to reporting on it in November. Gail?

MS. HUNT: Gail Hunt. I just wanted to be sure that there's a demand for this education and that it's not just an idea that we're coming up with because we think it's so exciting and --

CHAIRMAN NORQUIST: I think there's a demand. I think all of us who teach --

DR. EPSTEIN: Harvard put together what's called a MOOC, which is a massive online voluntary course; they had something north of 60,000 students take the course.

CHAIRMAN NORQUIST: I mean, and if you do this --

DR. EPSTEIN: It's huge.

MS. HUNT: [Off microphone.]

DR. EPSTEIN: Sorry, statistical methods for research.

MS. HUNT: Okay.

DR. EPSTEIN: Two good teachers put it together. They follow homework.

CHAIRMAN NORQUIST: I wouldn't worry about the demand. There's plenty of demand for it. Yes, Rick?

DR. KUNTZ: Just real quickly. I'm going to make some methodological geeky statements that -- Rick Kuntz here on the Board. You know, I thought the initial idea about the PPRN and the CCRN was to take established researchers with established methodologies and put them -- you know,

novel connection between patients who had very interesting patient-centered questions, and while I was very impressed by the ambition of the initial panel there, I thought that what they were doing was actually novel methodology and novel patient part.

It appears to me things are in flux here. So I want to make sure that your methods group is following this very carefully, but the idea of potentially doing the first throw network by leveraging EHR records and claims data to do research is not what I would call traditional research methodology. I would say that the way to do it is to actually have established networks using case report forms and typical methods to work with patients to get data out there faster.

But too many experimental methodologies and ways of combining patients' research, I think, is too much flux and I think what I'd like to know is, are you really engaged with the CCRNs with respect to them hitting the ground ready, methodology that's going to be ready for

publication and methods that have high levels of quality, completeness of ascertainment, and standards that are used for publication?

So, again, I don't want to distract from any of the enthusiasm that I saw earlier today, and I think it's really great, we need that kind of research going forward, but we want to make sure that we have the first set of the network usable research methodology that will get out the door in 18 months, 20 months, 24 months or whatever.

So, while it's not very popular to say that, I just want to make sure that we have at least some parallel processes in place that use traditional methodologies for research and data acquisition. And, again, not to undermine the extremely interesting, ambitious role to get data from other sources -- too many experiments might end up with no research at all.

CHAIRMAN NORQUIST: Thanks. Okay, Robin? Anything really critical? Because we're way over our time here and I'm trying to get us back on track?

MS. NEWHOUSE: No, thank you. I would say, great feedback and we look forward to reporting in November.

CHAIRMAN NORQUIST: Thank you very much, Robin. And thanks to the staff and to the Methodology Committee. Ten minutes and then we're back and we'll have to make the next section go, but it's an important section also. Thank you.

So, for people on the phone, we're taking a 10-minute break and we'll be back in 10 minutes.

[Recess.]

CHAIRMAN NORQUIST: Okay, it's time to start back. Joe? Okay, the next section -- we're going to start. You ready? Gail's here. You're ready to go.

So, the next section is on improving the Healthcare Systems Program, and basically what we're doing here is Steve -- they'll come in.

Steve, why don't you go ahead and sit down and we'll -- all right, I'm going to try this again.

So, the Improving Health Systems Program,

what we're doing here is Steve is going to talk about the program from the PCORI perspective.

We've asked Rick Kronick to come and give us his perspective on what AHRQ is doing. Once we've got these discussions from both, then we want to have a general discussion about what our niche is in this area, what do we think we should be doing a la what AHRQ is doing and perhaps others.

Okay, so, Steve, I'll let you go.

DR. SELBY: So, if I could just say one thing first and that is that from the day that Rick Kronick arrived at AHRQ, we began conversations about the respective roles of AHRQ and PCORI. It's an area that really needs to be articulated well for a lot of audiences, people across the country ask, you know, what do you do and what does AHRQ do, and particularly since, as Rick was saying this morning, Congress transferred the responsibility for CER to us and really made it very clear that dissemination and implementation, among many other responsibilities, lies with AHRQ.

And there is no -- so, we meet really

quite regularly and lately Jean Slutsky and I have met with Rick and Bob Kaplan from AHRQ at least monthly to discuss these issues, and there's nowhere where the potential for confusion is greater than with our Improving Healthcare Systems program and with the work that AHRQ does. So, it's fortuitous, but ultimately you could say it was planned after we realized that it was happening, that Steve Clauser, who joined us in January as the Program Director for Improving Healthcare Systems Program, he came from NCI where he did a similar kind of work around systems and quality and performance in cancer, will talk about how we apply the CER framework to Improving Healthcare Systems.

Rick will talk about the PCOR Trust Fund Portfolio and its emphasis on quality and safety and dissemination and implementation, then I think we could really have a fruitful discussion about what we see as our role and how, in fact, we do work closely with AHRQ to complement and not duplicate.

So, thanks to both of you for being here

to present today.

MR. CLAUSER: Well, thank you, Joe, for that introduction. I just want to say that in the interest of time to help us get going, there are some examples in my slide deck that were made available to the Board before. I'm going to kind of go through those fairly quickly and if there are questions, I'd be more willing to talk about that at the conclusion of the presentation or after Rick's presentation.

What I'm going to do today is talk a little bit about who we are, our staff, the goal we've set for improving healthcare systems research as well as some of the conceptual underpinnings of that research, trying to respond to what Joe was talking about in terms of how we actually do our work, and then talk about what we do in terms of both investigator-initiated contracts we've awarded as well as more stakeholder-driven priorities that are now taking a lot of our time in recent months, and also a little bit about what we've learned, we've had a few projects that have gone into the

second year of operations and a few of our operational observations from that viewpoint, and then where we're going, at least in the next 12 months, to try to improve the overall effectiveness of our program and supporting the PCORI scientific mission.

This is our Improving Healthcare Systems team, we're small but we're highly dedicated to advance our IHS goal statement. I'm not going to read this specifically, you can kind of look at it on the slide, but I want to make two points about it, which I think are important.

First of all, when you think about other kinds of programs that sponsor health systems research, our program is very distinctive in the fact that everything we do is comparative effectiveness research, that is, interventions either compared head-to-head to another intervention or compared to well-defined usual care, and second, that these interventions are targeted to improve outcomes that matter to patients, and that really is the patient-centered

focus of the research in our program.

This slide really acknowledges our research program, that help organizations intervene on multiple levels of the health system to try to improve those outcomes that are really important to the patients that they serve. Our portfolio is no different than that and if you look at the middle column, I've laid our funded studies by where in that particular level their particular project is focusing their intervention.

And I think there's three take-home messages from this particular slide, first of all that about two-thirds of our funded projects either focus on interventions directed at the provider or healthcare team or the organization and practice setting; second, the topics that are typically used in our portfolio really cluster around two types, one, our projects that are targeted towards individuals with chronic diseases, in fact, often multiple co-morbidities. Those are the complex patients whose goals healthcare systems really have a challenge in supporting, and also a lot of care

transition projects where we were looking at the challenges of moving patients from one delivery setting to another throughout an entire episode of care. And finally, one thing I think that's important to note about our projects is that most of the comparators, as you can see in those examples, are really interventions compared to well-defined usual care. These are the majority of the comparisons that are currently done in our program.

We have been working with our Improving Healthcare Systems advisory panel over the last couple months to begin developing a strategic framework to help communicate more effectively to the investigator community and to other stakeholders exactly the kind of work that we do in our program. This is a work in progress, but I think it's helpful to try to illustrate what we're doing.

On the left hand side, that box deals with the foundation for our research and that is the nature of the evidence-based interventions that are

used in studies. These interventions must be evidence-based and they reflect four different categories of intervention types ranging from technology to personnel and workforce, to incentives, and organization structures and policies.

And then if you go on the far right, the other distinctive feature is that these interventions are supposed to be targeted to improve those outcomes that matter to patients, and that typically goes beyond some traditional health systems quality improvement research, it looks at things like utilization and mortality to also look at patient-reported outcomes, those outcomes that deal with care experiences, deal with physical and emotional functioning, issues that are very important to patients in characterizing their experience in receiving healthcare from health organizations.

And because we are a health systems research organization, all applicants that make application to our program really have to identify

what is the system failure that your intervention is designed to address to improve these outcomes and how, in the course of those comparisons, will we learn that practice will in fact be approved around some of those characteristics of improve practice that we have listed on that middle slide.

And finally, I just want to say that this is a -- not a static process, it's a dynamic process in which patient and stakeholder engagement is throughout the whole research enterprise, which we hope will fundamentally alter the nature of the research to really improve outcomes that matter to patients, and thereby, change practice.

So, the distinctive components of our IHS studies are that we adapt our PCOR model for CER, beyond clinical treatment options to different levels of the healthcare system, but we require inclusion of well-articulated and valid comparators both for trials and studies using observational data. We focus on outcomes relevant to patients, including patient-reported outcomes, but we have active involvement of patients and other

stakeholders throughout the entire research process in studies that really are done in real life settings to try to maximize its potential for sustainability and scalability.

Now I'd like to shift gears and talk a little bit about our current portfolio, and do that in terms of it's now our current foundation in our IHS program. These are studies that have been funded to our IHS broad PCORI funding announcement.

These are the parameters of our broad PFA. They involve all studies, involve comparative effectiveness of alternative features of health systems, but the priorities that come from this announcement really reflect investigator interests, merit review assessment, and programmatic balance as we continue to build our portfolio.

IHS has participated in all five broad funding cycles that PCORI has supported and that has resulted in 48 investigator-initiated studies. Our investigators are now working in 17 states and the District of Columbia, and accumulated about -- a little over $90 million in awarded projects.

A word about our study designs, almost three-quarters -- if you look on the left in the larger circle -- about three-quarters of our study designs are randomized clinical trials and of those a little over half are multi-site trials, and if you look at the smaller circle on the right hand side in the green quadrant, you can see that about a third of those studies are cluster, randomized designs.

We have found it useful to kind of distinguish our portfolio by the IHS projects that really target single system interventions versus those that deal with multi-component interventions, and this slide shows that a little less than half of our projects really are focused on single system interventions and comparisons related to the PCOR research.

Of those 22 projects, a little more than half are focused on organization structures and policies. These are projects that are really focused on clinical redesign, such as the development of multi-disciplinary teams to try to

improve comprehensive treatment planning and treatment follow through, or projects that are collaborative care models where behavioral health is brought into work in primary care disciplines.

Now, the next cluster of projects on the right hand side in the green box are technology projects. They tend to be of two types, projects that really involve web-based interventions of a variety of type which really try to engage patient communication, engagement, or access to information or services in a health system, or telemedicine, which oftentimes is bringing specialty care to individuals who live in rural areas.

And finally, our personnel interventions tend to be projects that really kind of augment existing clinical systems by adding such things as patient navigators to facilitate access for patients to information or services that are difficult for them to obtain.

Like I said, there are a couple examples I have here, one on organizational interventions and another one on technology, which were in your Board

packet and they were just examples of how these projects were utilized to support the work that we do, but I'd like to switch now to projects that have multi-component interventions, this is more than half of our portfolio, in which they tend to distribute into about half the projects being involved in technological interventions, whether it's web-based technology or even telemedicine, where additional medical personnel or patient navigators are brought to augment that intervention to facilitate patients or sometimes even clinicians in using that technology effectively.

Other types of interventions on the right hand side, in terms of organizational interventions, in multi-component research these tend to be interventions where the healthcare systems bring considerable more resources in the example such as additional personnel where they're doing such things as taking elements of the patient-centered medical home model and putting it into specialty care to look at the comparisons of specialty-based home care models versus other types

of comparisons.

And I also have a couple additional examples here. This one is particularly interesting because it is the only intervention that really focuses on the use of patient incentives as an intervention in our research, and the other one was just a note that we do have interventions that do deal with multi-component interventions where we can actually begin to disaggregate some of those interventions research to find out, are there aspects of the multi-component model that are really driving system change to see if, in fact, we might be able to settle for some less complex, lower intensity interventions that might accomplish a lot of their goals, and we would like to see more of these types of projects in our portfolio.

That was the -- kind of a brief overview of the work we have done in our PCORI broad funding announcement, now I'd like to shift gears and talk about our stakeholder-initiated priorities because this is an area where, I think, we're very

interested in expanding our research portfolio in the coming years, in particular because these allow the opportunity for larger, more impactful studies in health systems comparative effectiveness research.

The first project is called STRIDE, that's Strategies to Reduce Injuries and Develop Confidence in Elders. This is a Board-directed priority that PCORI is partnering with the National Institute on Aging and in June of this year we awarded a $30 million five-year award to investigators who are really recognized as some of the real national experts in falls prevention research in the United States. We have -- it's a large trial, multi-cluster randomized trial of 6,000 participants involving ten different sites, 80 local practices, which really reflect the diversity of the types of health systems, the types of geographic context, and the diversity of populations within the elderly who are at risk for falls face across the United States.

The intervention is a nurse falls care

manager who works in collaboration with the individual's primary care physician using a bunch of evidence-based, individually tailored services to really tailor a program that really meets the unique needs of every individual that they are serving, and the comparator for this study is primary care, but primary care that's been enhanced to have formal falls risk assessment as well as evidence-based patient education materials.

The last example I'd like to propose is our effectiveness of transitional care initiative. This was a topic that was identified as a high priority by the IHS advisory panel and it really requests investigators to compare which transitional service clusters are most effective in improving patient-centered outcomes. It really reflects to a lot of systematic research that says we really do need to know exactly which types of service bundles and programs would really work for what types of patients under what types of contexts.

This is a large initiative, a $15 million

study that will go to one awardee. We will be presenting tomorrow to the selection committee our funding slate on this and we hope that we will have an award to announce by the end of the month. And I just wanted to emphasize that we're very proud of this fact, that this is the first prioritized topic by a PCORI advisory panel to complete the entire targeted PFA process.

Finally, I just want to remind you that we do -- our program does participate in the pragmatic clinical studies PFA program. We have four specific defined priority topics that are included in that funding announcement that were vetted by our advisory panel and we're very hopeful that we will get some very large, impactful studies from this initiative to, again, complement our movement towards targeted and larger projects.

So, in conclusion, we have developed a portfolio of comparative effectiveness studies that really do include intervention comparisons to either head-to-head or with well-defined usual care, but we still have some work to do to continue

to improve this program.

I mentioned earlier that we do have a couple studies that have now transitioned into their second year of operations and some of the early lessons we're learning is that these studies are complex in health systems research, and the clinician and administrator buy-in is essential for these studies and it's especially important when these interventions involve clinical redesign, that are asking clinicians to do work differently than they have done before, and also, and this is not, I think, uncommon to a lot of other programs, is that accrual of understudied populations are challenging in these studies.

And I think one of the lessons we've learned is that we have to do much more closer contract management and proactive communication with investigators to minimize the risks of these studies falling behind.

And finally, I just want to identify some of the goals that we've kind of set for ourselves moving forward or over the next 12 months. First

of all, even though we are trying to move to larger, more targeted and impactful studies, we do value the work that is being done under our broad PCORI funding announcement programs and we have initiated a number of initiatives over the last year to really try to improve the applications we get and programmatic fit. For example, we have gone through two cycles of competitive screening of letters of intent to try to improve the overall competitiveness of applications to go to merit review.

We are enlarging our project applications. We now have a broad option in the program for people to solicit up to a five-year $5 million broad PFA application and that is above the $1.5 million and three-year limitation that we currently have.

And finally, we have been trying to communicate areas of emphasis within our broad portfolio to try to steer our portfolio in ways to emphasize projects where we see needed gaps or particular areas that are of high priority.

And in addition to all this, we do want to continue working with the Methodology Committee and Clinical Trials Advisory Panel. We're very excited to hear about the work that is currently being done on cluster randomized designs as well as complex interventions, because as you heard before that, it's directly related to many of the studies that we support and sponsor in our program.

And finally, we want to develop new initiatives with other PCORI programs. We have an activity under way, for example, with Communications and Dissemination Research to look at the appropriate use of evidence in health systems and communication research that addresses programs like, for example, Choosing Wisely, and also begin conversations with PCORnet around the potential applicability of Health Systems Research in a network like that, taking advantage of the very exciting meeting we had at IOM recently to look at the prospects for thinking about comparative effectiveness research of rapid cycle improvement.

And finally, I just want to say that as we get to know our portfolio more, we'd really like to engage with our other funders around kind of clusters of IHS projects where we see potential implementation challenges that could be good areas for implementation research by organizations that sponsor that type of research and hopefully in doing so, in those collaborations, maybe identify other CER questions that we can benefit from in our own program development. Thank you.

CHAIRMAN NORQUIST: So, let's take a few questions here about our portfolio and then we'll let Rick talk about AHRQ's.

So, Rick Kuntz, you were up first.

DR. KUNTZ: Yeah, first of all, fantastic presentation and when I was watching your presentation I thought, you know, the problem with CER is that it's difficult to understand whether or not you need to compare a new therapy against a standard of care or actually compare to active therapies and there's always this issue about the fact that, you know, typically in industry we take

this kind of standard of care, which is so baseline that anybody can win if you do an intervention, but that's not really the question people are asking.

So, I looked your falls, if we said the standard of care is nothing at all, that's not really a nice study to do because we have a problem already identified, this is a problem we had to address, so you're looking at two active approaches.

It would be interesting to be able to put a framework together to say when you're doing CER that, number one, you would establish that whatever you call the standard is not acceptable, and that's why you're looking to active therapies, and then contrast active therapies with -- one is more intense than another, what are the intensities between them, because I think the two that you contrasted, one was a very intense therapy and one was a less intense therapy, but if we had a framework that said, okay, what are you getting on the CER part?

Number one, is there an established

baseline, is it acceptable, number two, are the competing therapies contrasted based on costs and so on, would be a nice framework to have and I think that when you look at your transition care, that was more of establishing kind of a standard that's out there by some practices versus a new intervention, and I think that we've become more familiar -- because you might be able to come up with a novel framework to put that together, but I thought the studies were great, it's just that in order for us to put it in context, it might be interesting to develop a general framework for why you pick certain -- you know, randomized therapies to conduct.

MR. CLAUSER: Yeah, most of -- as you know, most of the work that is in our portfolio really does come through our merit review process and we're working very closely to try to, number one, begin to be much more clear in our funding announcements about the specific kinds of research we want and we're working very closely with our colleagues that run our merit review program to try

to communicate those changes.

And I think, you know, as I mentioned, kind of our strategic framework was kind of an -- beginning of an evolution of that and I think kind of where you're taking it to that level is something we definitely should consider and trying to give very clear guidance to the investigator community what we really want to look at in our program.

DR. KUNTZ: Basically I want to leverage your great work to see if you can develop a common general model and that would advance the field of CER.

DR. DOUMA: I want to thank you very much. It's exciting to see how you slice and dice and analyze your information and what you're doing, presumably to help you figure out what to do next, and that without that it's difficult to make that next decision.

But I want to ask a question, in developing new initiatives with PCORI, you have listed communication and dissemination, and in

listening to our discussion about the dissemination of the guidelines, would that be a project that AHRQ would like to get engaged with us in order to better disseminate guidelines?

MR. CLAUSER: I think that's an excellent question to ask Rick, you know, when he has his presentation about what work they are doing in guidelines, you know, I think that would really be for you to respond to when you're ready.

DR. LEVINE: Yeah, so it struck me, a quick question that the active intervention for the falls, is that a registered nurse as a falls manager?

MR. CLAUSER: Yeah, that could be either a registered nurse or even a nurse practitioner depending on, you know, what the site is willing to invest in.

DR. LEVINE: Right, so from the scalability perspective, that's going to be a very expensive intervention. I think you can predict that, you know, people know that the more intense and individually focused intervention, the more

likely it is to show benefits. And I guess it just makes me nervous when we think about scalability as something -- editorial comment, but in terms of being able to implement the subsequent findings should it produce what I would expect, which is we see an improvement in outcomes based on the level of intensity of intervention, potentially anyway.

But the other -- my other comment is about the portfolio. I think it would be interesting to look at this portfolio compared not to AHRQ or to NIH's portfolio, but to IHI, because an awful lot of what you've described are rapid cycle operational improvement projects that are going on in health systems around the country, integration of behavioral health, many of the topics that are in the compendium we saw are actually going on today as rapid cycle improvement projects in a site specific place.

So, I wonder whether there would be interest in looking and seeing who's doing these things right now.

MR. CLAUSER: Yeah, I think it's important

to understand that to me it would be great if the topics -- if we're taking high priority topics and those topics seem to be going around to organizations that have very different research functions. We are not in the business of doing quality improvement research. As I mentioned, our research is really comparators of interventions, head-to-head to other interventions or to well-defined usual care to really provide patients options and understanding which options of system innovations work for them and meet their needs, and that's very different than what --

DR. LEVINE: No, I understand, but what I'm saying is, are we certain that these projects are actually comparative effectiveness research rather than a reconfiguration of an operational improvement project that has, you know, years of experience in doing things one way, they change the way they're doing things, and they look at the outcome. It's not real comparative effectiveness research that's happening, but if it's close enough that it meets the standard that -- what this

portfolio is doing is meeting the standard of what I think we intended with improving health systems or is it to micro focused?

I don't -- it's a concern I have and I don't have an answer.

MR. CLAUSER: I think it's a very legitimate question to ask about the portfolio because as you saw on the one slide, you know, our projects are intervening at very different levels of the system to try to figure out, you know, which types of comparisons target at what level of intervention in the system is really going to lead to the best outcome.

The other point, though, the interventions that are in our portfolio, you know, they are evidence-based, they are based on some prior work, either pilot work that are well available in the community so, you know, they're building off work that has been previously done, but trying to do it in a very focused, experimental, rigorous setting.

DR. SELBY: So, Sharon, you're asking exactly the right question and I think it's a

question that we ask ourselves and it's a question that we ask in our meetings with Rick and Bob as well. The Board, in its wisdom, identified a priority for CER called Improving Healthcare Systems and we have been applying it for the last two plus years and it is a subtle distinction and we are wide open to working with the Board to refine our vision of comparative effectiveness research, how can CER apply to healthcare systems.

I'll just note that the IOM 100, 50 of them are systems-based questions and they took great pains to point out that much of CER should be about improving healthcare systems. The FCC CER did the same thing, said, by the way, this is not just treatment A versus treatment B in systems. But, you know, when you get to performance improvement, quality improvement, and what we do, we could use perhaps a better operationalization of how we select what we select and how we solicit what we solicit.

DR. LEVINE: Yeah, so I guess -- and I was one of the strongest advocates after Arnie insisted

we do this that this be part of the portfolio and I guess I'm struggling with -- and I don't mean this in a demeaning way -- small questions versus big questions. So, how -- you know, emergency department functions in this country are a significant thorn in terms of delivering patient-centered, high quality care and they have evolved over time. Emergency care has evolved over time based on a whole lot of factors.

You know, is there some way that -- is there some thoughtful approach to looking at the organization and design of emergency departments in the entire continuum of care that would somehow improve the functioning, the efficiency and the effectiveness and the extent to which people got to the right place to get the right care at the right time? To me, that's a big question that has national implications compared to should behavioral health services be collocated with primary care? I mean, that's kind of a no-brainer to me.

But, anyway.

DR. WEISMAN: Sharon, I'm really

struggling over what you said because usually -- I mean, you're so articulate and you just nail things. But I'm really --

[Laughter.]

DR. WEISMAN: -- not getting it -- aren't we supposed to be improving quality? I mean, isn't that improvements in the quality of care that leads to improved outcomes for patients in a patient-centered way? Isn't that what we're doing? I mean, isn't that what we're about? And this also seems like it would have -- that if you do this in the right way, and I have no idea what's big or small, I mean, that's part of what research is. I mean, maybe things that we think are small actually have bigger impact.

DR. LEVINE: I'm not saying I'm right.

DR. WEISMAN: No, you usually are. I'm doubting myself. But I like what he presented. I thought this was great stuff.

You're also -- you know what, you're in a health system that's less fragmented than most and maybe you don't experience what a lot of people

experience.

CHAIRMAN NORQUIST: All right, so we'll let -- Arnie wants to say something here and then we'll --

DR. EPSTEIN: Sure. So, I have the same question as Sharon and I wonder if we'll learn a little more after Rick speaks in this way. He's going to talk about a big project they're doing and the endpoints are HSR endpoints more than PCOR endpoints, but I think they don't have to be. They could be PCOR endpoints because PCOR is not so different than HSR at some root if you think about what's on the left hand side, and I think he's trying to do some stuff which is bigger and more imaginative than we've been able to do in part because he's going to make a bigger bet on it than $2 million times 14 projects.

CHAIRMAN NORQUIST: So, let's let Rick -- and then we'll come back.

DR. EPSTEIN: At the beginning and at the end.

MS. HUNT: While he's getting up, I'd just

like to second what Sharon said about scalability, the concern about scalability of the falls prevention.

CHAIRMAN NORQUIST: Oh, okay. Thank you. Okay, Rick.

MR. KRONICK: Thank you for the opportunity to talk about what AHRQ is doing, the portion of the Patient Centered Outcomes Research Trust Fund that comes to AHRQ, which as I'm sure you all know is 16 percent of the trust fund.

Before I go into this, just a kind of comment on the last discussion and kind of the points that Sharon raised, you know, we talked earlier in the day about the need to focus and there was kind of general agreement about the need to focus, although not agreement about focus on what, and I think the last discussion is interesting in that regard and it would be useful, I think, to come back to the question of whether the sorts of studies that we heard about are kind of part of the focus that makes sense, and I think part of the question for me is -- and this will get

more directly into the presentation I want to make -- is around imagining the pathway from the results of the studies to change the matter for patients and how the results of an IHS study will get implemented by health systems. Will it make any difference for them? And that's a really hard question to answer and I'm not going to answer it in the next 15 minutes with the work we're doing, but I hope we can come back to that.

So, I want to spend a few minutes on an overview of the agency, because some of you -- many of you are quite familiar with the agency but some of you may not be, and then talk about the work that we're doing in patient-centered outcomes research, which is only a part of what we do in the agency, about 20 percent if we look at the funding levels.

This slide is our mission, I'm not going to read through it, but we are an evidence producing agency, our mission is to produce evidence; to produce evidence for what? To make healthcare better and, you know, higher quality,

safer, more equitable, et cetera, and then it's got this long and kind of difficult parenthetical, and to work within HHS and with others, try to make sure the evidence is understood and used. I said I wasn't going to read it but I actually knew that by heart, I wasn't reading it.

And this is a reminder particularly to me and to staff that just producing evidence doesn't matter and the point is to try to make sure that this evidence is understood and used.

We are a relatively small agency, about $460 million of annual budget, 300, more or less, FTE employees. So for a research agency we're really big, but in the context of the health system, obviously, we're very small. We do have lots of partners, particularly within HHS, but also outside, to try to make sure that the evidence really is understood and used.

We have four priorities -- quality, safety, access, and affordability. You know, it's this narrow agenda, and then we have kind of equitability scattered as part of all of these. We

don't have a separate priority on trying to make healthcare more equitable, but in each of these four areas we try to work and focus there.

Our grant activity in the agency is about $170 million. It varies by year. You know, I said earlier, about $460 million all together, so there is about $70 million or so in program support that the combination of the two things that Regina showed earlier, administrative support and program management, so that's about $70 million. The rest that's not in grants is in contracts, so we conduct the medical expenditure panel survey, which is a family of surveys, large household surveys, as well as a survey of employers.

We produce the healthcare cost and utilization project data, a data set that has information on 97 percent of every hospitalization in the country. We do a lot of contract work outside of the grant portfolio. You can see in this pie chart that the PCOR part of the grant portfolio is, in '15, estimated to be about $66 million, so a little under a third of the total

grants.

The purely investigator-initiated grants, about $46 million, so this is a very broad funding announcement in which we say we're interested in safety, quality, access, affordability, provide a little bit more context around that and see what comes in the door, and go through a peer review process.

The other large segments here are grants that are targeted at producing evidence about improving patient safety and grants producing evidence about how health IT can be used to improve safety and quality, and these come in line items as part of our budget.

I am not going to go through this slide. It's got the four priorities in the four corners, a smattering of AHRQ activities in the rectangle with the activities trying to be somewhere closer to the priorities that they are directed at, although of course many activities are directed at producing evidence for multiple priorities.

So, just a couple of things to mention

here, I discussed MEPS, the Medical Expenditure Panel Survey, there's not a laser pointer but it's sort of down in the bottom, a very large activity in the agency, about $65 million in contract work, again, it's a household survey of about 30,000 households and it's the major source of information on what kinds of healthcare gets used by whom and how much gets paid for and the sort of "so what" that's used by many researchers and analysts and the Congressional Budget Office to try to figure out what's going to happen when subsidized health insurance is made available to people, and actually I think one of the sort of remarkable, unsung stories, is how close the CBO got to estimating what enrollment actually was in the context of tremendous uncertainty. You know, they use MEPS.

The CAHPS, the Consumer Assessment of Health Plan, in the sort of middle left, is a family of surveys that have been developed by the agency and now many other people to gather information from consumers about the experience that they have with physicians, hospitals, home

healthcare agencies, nursing homes, many other settings of care.

On the upper right, CUSP, the Comprehensive Unit Safety Program, I believe, is a set of contracts to implement in primarily hospitals, although we are expanding to nursing homes, evidence that's been generated about how to make healthcare safer. So, we funded, through investigator-initiated work over ten years ago, Peter Pronovost, who did work in the State of Michigan showing that, you know, very simple approaches to making ICUs safer could reduce central line infections, and this work, through the CUSP programs, and then more recently through work that CMS has done and the Partnership for Patients, that led to about a 40 percent reduction in central line infections.

We have more recently been working on catheter associated urinary tract infections, other surgical site infections, other kinds of adverse events. We are expanding this work, as I said, out of the hospitals into nursing homes and moving into

ambulatory settings, although much more difficult, particularly trying to work on diagnostic errors.

Let me move now to -- closer to what are we doing with the trust fund, but before I do that a couple slides on kind of history of AHRQ's involvement in comparative effectiveness research. So, the Medicare Modernization Act of 2003 created the effective healthcare program, under which the agency reviews -- systematic evidence reviews generates new scientific evidence, translated research findings into formats that patients and physicians and other clinicians would find useful.

ARRA provided a large infusion of funds for this program. I'm not going to go through this slide, but mention the second down on the left, the CHOICE program, and I mentioned earlier today in the closed session, you know, a couple of the findings that came out of that on corticosteroids showing that six weeks out -- and I think this is maybe the next slide -- that patients with lumbar stenosis who receive an injection of corticosteroids along with an anesthetic, don't

seem to be doing any better than patients who just received an injection of the anesthetic.

In a somewhat similar vein this week the New England Journal next week will be publishing the results of a study comparing ultrasound with CT scans for patients who present at the emergency room with kidney stones. These came out of the CHOICE work. That is work that we do not fund anymore.

So the steroid study -- PCORI is funding a follow on to that looking at what happens to patients 12 weeks out. That was work that was an important part of what the agency funded. With the establishment of PCORI Congress has said PCORI should fund that work and has said to the agency that they're not interested in us funding that work, and we were delighted that PCORI is doing that.

What are we doing? Well, we are charged with figuring out how to train researchers in conducting patient-centered outcomes research, and importantly, figuring out how to effectively

disseminate patient-centered outcomes research and to disseminate what is known to work.

We have four main activities in this area, training -- and I'll talk briefly about each of these, although more about the last -- we work on training, on producing systematic reviews, on translating what's known into something that can be used -- that will be useful for patients and clinicians, and then broad-based implementation and dissemination, which is -- all of these are hard. I think the last is the hardest.

Training, we fund an alphabet soup array of K08s, R24s various numbers, institutional training grants, individual awards at various levels of training. We put about $20 to 25 million a year of the PCOR Trust Fund money into training researchers in how to conduct patient-centered outcomes research. There's one example of this -- listed on the slide that Robin's been involved with, I believe.

We are looking also at trying to ask the question of whether the -- our training efforts

should be encouraging even more innovative methods of training researchers to do PCOR, particularly trying to incorporate some of the kind of systems engineering in quality improvement approaches as well as big data work that, you know, some of these institutions that we are supporting or working on, but more needed here in the future and things that we are looking at additional developments in the training area.

The second major activity is the production of systematic reviews. As part of disseminating patient-centered outcomes research, it is rarely one study that results in changes in practice, and so we have invested in the production of systematic reviews as a basis for work that would then be disseminated -- the dots here show the locations of the current contractors for the evidence-based practice centers. We produce around 20 or so of these systematic reviews per year with some variation from year to year.

The third main activity is translation of PCOR findings into something that can actually be

used by patients and physicians. The systematic reviews that I showed on the last slide in the New England Journal articles are not something that are useful to patients and often not even so useful to physicians. And so, the Eisenberg Center translates the results of PCOR into clinician guides, patient decision aids, consumer guides, and makes this information available to patients and clinicians.

There are about 3 million pieces of information that get distributed a year.

But making this information available is, while necessary, not sufficient to accomplish change. You know, this is a slide that comes out of the National Healthcare Quality Report that gets produced by the agency every year. Many of you, I'm sure, are familiar with the work that Beth McGlynn did probably more than ten years ago showing that, you know, only about half of recommended services were received by Americans.

This slide uses a somewhat different set of indicators so that 70 percent should not be seen

probably as an increase from Beth's 50 percent, but it's a different set of services.

There are so many services that only half of Americans receive even though we know they work. I'm going to talk in a minute about cardiovascular risk factors. You know, Mike Lauer is here, I'm embarrassed to say this in front of him, he knows 20 times more than I ever will, but close to -- only a little more than half of Americans with high blood pressure or high cholesterol have it controlled. And we know at a sort of technical PCOR level how to do that, but it's not in practice, and so the kind of challenge that we see is how to disseminate and implement PCOR in a way that really works.

One slide on some of what we have been funding in this broad based dissemination space, Partnerships for Sustainable Research and Dissemination, in which we are working with grantees -- and both the first and the third are actually directed primarily at underserved populations. The second is working with a large

variety of specialty societies to try to enlist their help in getting information disseminated.

We are moving, then, to projects that I hope will have a more -- a clearer link to being able to show that we're effectively changing the information that physicians and patients have and the practices of the way that healthcare is practiced. So, we issued a funding opportunity announcement in March, are currently getting ready to review proposals. We got a very robust response, looking for up to eight grantees around the country who would form regional cooperatives to try to figure out how to -- what sorts of supports small- and medium-sized primary care practices need to be able to improve performance on cardiovascular risk factors.

We've seen in some of the larger organizations around the country -- Sharon's here from Kaiser and some others -- that there are a variety of large healthcare organizations that have made progress in disseminating and creating systems in which the results of patient-centered outcomes

research get implemented to improve outcomes for patients, but that many small- and medium-sized practices have had a much harder time figuring out how to do this effectively.

And so these grants, which would be up to $5 million a year for each of these up to eight regional cooperatives, so for us a very substantial investment, looking at a three-year program, would be charged with trying to figure out how to improve these outcomes and then more broadly how to -- how these practices are better able to incorporate new patient-centered outcomes research as it emerges so that the sort of proximate goal is around cardiovascular risk factors and incorporating PCOR evidence there, the larger goal is having these practices being better able to incorporate PCOR more generally.

We issued a companion FOA for an evaluation of this work. Each of the grantees would be expected to have substantial evaluation efforts as part of their work and then an overarching evaluation that also will be going

under review that also had a quite robust -- there's a lot of interest in it.

The second initiative that comes much closer to the discussion that we just -- that Steve just led us through is an FOA that was released in July -- early July in which we are interested in funding up to three centers of excellence who would study how health systems are disseminating PCOR, and increasingly care is being delivered by health systems.

Although a little part of the problem is we don't even have a very good definition of what health systems are, so that might be part of what these centers would work on, but we know very little about how health -- which health systems -- what health systems are doing to effectively disseminate PCOR work and these three centers of excellence would be -- I hope, be working on trying to figure out what the various systems are doing to disseminate PCOR, which of these efforts seem to be effective and successful, and how the dissemination of PCOR within systems is related more broadly to

measures of health system performance on things that matter to patients -- quality outcomes as well as affordability and cost.

The applications here are due in the middle of October.

So, these last two initiatives are bookends on our dissemination work, one that's focused on figuring out how to disseminate PCOR in small- and medium-sized practices and is clearly an intervention project, and it is, by our scale, you know, a pretty large scale intervention project, it's -- we're targeting about 6,000 primary care physicians, would be taking care of nine million patients, we imagine, if the panel sizes are 1,500 per physician.

So for us it's a pretty large scale intervention and dissemination project, but still clearly a very small part of 330 million Americans, and [microphone hit - inaudible] physicians, so the production of evidence part we see as key, that is, this will be successful in part if we improve blood pressure and cholesterol control for these nine

million patients from 50 percent to 75 or 80 percent, that's an approximate measure of success, but then it's really only going to be successful if the evidence we produce gets used by CMS, by other payers, and by other folks who are involved with these small- to medium-sized practices to actually institutionalize and disseminate it more broadly.

What we realize is that even with the resources we have, we probably can't effectively disseminate to 200,000 primary care physicians in the country or 330 million people, so the production of evidence is key, and then the bookend part is that we also want to understand what large systems are doing in dissemination, what they're doing that's effective with the thought and hope that if we produce evidence on that, that that will then be useful potentially for payers and for systems, certainly, to try to figure out how to improve patient outcomes moving forward.

And then the last -- to mention about our use of PCOR Trust Funds is that we are charged in the statute with facilitating incorporation of PCOR

into clinical decision support tools and we are working on figuring out how to implement that piece now.

So, in summary -- and I know we're kind of over time -- but our work in PCOR has pivoted to dissemination and training. I tried to describe earlier that, you know, prior to the establishment of PCORI, we were doing work in generating PCOR. That is clearly PCORI's responsibility and also with the end of the ARRA funding, we would have had limited funding for that in any case in the absence of the resources that are now available in the PCOR Trust Fund.

There is a great deal of excitement in the agency about this pivot, although as you can imagine, also change is always difficult, and as Joe said, we are meeting very often, at least monthly if not more, with Joe and Jean and others at PCORI. Clearly, you know, production of evidence is necessary but not sufficient to get used and we are working on trying to figure out how to get it used.

So, I will stop there and --

CHAIRMAN NORQUIST: Thanks, Rick, very much. That's very helpful to see where you guys are at this point.

So, what we're going to do is we'll spend about ten minutes for questions. We have to break at 5:15 in order to have the public comment period and then we can come back further, so we don't want to just cut it right off. So, I want to allow people to go. So, we'll go this way. Mike?

DR. LAUER: Rick, that was great.

CHAIRMAN NORQUIST: Wait a minute. I'm sorry.

DR. LAUER: Mike Lauer, Board for NIH. So, Rick, that was great. Thank you so much for the update and just a clarification. So, the ABCs project, the Million Hearts project, that's coming out of PCOR Trust Fund money? I'm sorry if you already said this, how much money is going into that?

MR. KRONICK: We haven't made awards, so it's not yet clear, but we have said that it would

be up to $40 million a year for the grantees from the regional cooperatives and then an additional up to $5 million in an evaluation contract.

DR. LAUER: Excellent. Thank you.

DR. KRUMHOLZ: Thanks very much. I think that's really helpful for us as a Board, and you sitting in both places can help us. I think we ought to really try to be sure that we're not overlapping too much and that, you know, there's really complementarity between the efforts that you're making and the ones that we're doing. So, I don't have an answer to that, but it just seems to me that we have to work hard to make sure that we're hand-in-hand, which is in part why you're presenting now, and I think we ought to be thinking about that with regard to our prioritization strategy.

With regard to the field in general, just because Steve and Rick presenting, I think one of the tricky pieces here is the evaluation of the evidence and also the fact that these are complex interventions, which Robin has the Methodology

Committee thinking about, so that the scalability and generalize ability of these things and having it tailored and what is it that's essential to fostering performance and what is it that is unique to the particular setting is what's what one of the challenges in doing this kind of work, and we need help methodologically, we need to be pioneering this, because the evidence needs to be said; ABCs is a perfect example. Is -- you know, cholesterol targets are no longer parts of the guideline. So, we actually don't control cholesterol anymore, cholesterol -- we assess risk and then treat with statins, which is the evidence-based medication, and by the way, I don't even know if that will be rolled back in the next iteration of the guidelines.

So, it's hard to know, and so it becomes very tricky. At the same time, I know what you're trying to do is elicit enduring insights, generalizable insights that go across topics and so we need to keep moving.

So, anyway, I salute the effort. It's

tremendous. And our side, on the PCORI side, I think we need to be just very thoughtful about how we strengthen and complement the kind of work that AHRQ's doing and then, you know, Steve, help us think about where the gaps still are that we're filling given the work that they're already doing.

CHAIRMAN NORQUIST: So, I think that speaks to the need for very strong collaboration in what we do so that we're constantly having this discussion about that so we don't overlap, but we're also very clear about what our priorities are. They're pretty clear.

Steve, I'm sorry. I think I missed you. You have your tent card up.

VICE CHAIRMAN LIPSTEIN: This is a question and comment. You introduced us to a new acronym, FOA --

MR. KRONICK: Funding Opportunity Announcement, sorry. I've been in the government too long, clearly.

VICE CHAIRMAN LIPSTEIN: We use PFA, right? Right. Okay. My comment is obviously

nothing takes place in isolation. Right now, what's happening in American healthcare is this huge up-consolidation of the delivery system triggered over the last six years by everything you already know about, and that up-consolidation is bringing together community help, then you use with academic health venues, with inpatient and outpatient and home care across a continuum so that you've got almost all these alternative settings involved in something that's beginning to be under more corporate umbrella-like control.

There's got to be an opportunity in that for dissemination of PCOR. I just don't know how it plays out, but as you think about dissemination and you think about this up-consolidation movement, there's got to be something in here that works to our advantage, and so I just wanted to plant that seed.

MR. KRONICK: I agree with you completely and the PFA that we release in July or June is exactly trying to move in that direction, at least trying to first understand what various systems are

doing and -- as a way of an entre of being able to figure out how to effectively disseminate two systems. I agree completely.

CHAIRMAN NORQUIST: Freda, you're up next.

DR. LEWIS-HALL: Freda Lewis-Hall, Board. I actually had two very different questions, one is, I'm struck by the work that you're doing to integrate some of this in educational programs, it follows onto Harlan's question earlier, and I guess I'm a little shocked that the answer wasn't, at least in part, AHRQ's doing some of that, right? So, I think that it underscores Gray's point about, you know, kind of integration and information sharing around the programs because I could, you know, kind of see the Methodology Committee, for example, going back and repeating some of the work that, at least in your synopsis, sounds like you might be working on. So, I'm not quite sure how that happens, but that was striking to me that you kind of had an outline of some things that we had talked earlier about as something that needed to be done.

And then -- or at least in part to be done.

And then the second thing is, and I apologize, because I came back in and you may have covered this already, what are some of the metrics that you're using around the effectiveness of the work? So, is it closing the gap on the number of people who are adequately treated for their hypertension in getting them to goal? Or are there some other -- you know, or metrics like that --

MR. KRONICK: That's a really good question, Freda. To date, the metrics have mostly been process measures around dissemination and contact, so, you know, are we getting information to physicians, to patients?

In the ABCS initiative, the proximate metric will be around improvements in cardiovascular risk factors. You know, as mentioned, the sort of ultimate metric will be, will the evidence -- well, the second level -- have we produced evidence about how to make these improvements? And then the real measure is, does

that evidence get understood and used and sort of picked up more broadly, so there are kind of a few levels here.

CHAIRMAN NORQUIST: So, I think -- that was a good point about especially the training and stuff that came up and I think to be clear about on what you're training or using our methods standards, for example, as part of your training or whatever. I mean, I think that's one of the things that we need to be very clear about so that we're not confusing investigators. We're trying to train them. One thing, you should certainly be collaborating on that.

Robin, I'm sorry, I'm going to let Rick say that but then we're going to have to stop for a minute and do the public comment as soon as you answer --

MR. KRONICK: Actually, I was just going to make two acknowledgments, one, you know, Jean Slutsky, who now works for PCORI, of course, central in a lot of the earlier work that we did and so if there are really tough questions I'll

turn to Jean, but Bob Kaplan, who's the Chief Science Officer at AHRQ has been working a lot on training and, Bob, I don't know if you want to say anything here about the training questions.

DR. KAPLAN: [Off microphone.]

CHAIRMAN NORQUIST: Yes, we can -- I'll repeat what he said, but basically that we are collaborating, you guys are talking with our folks here about what -- you have some R25 grants that you've just put out on this issue, but that we are -- it's good to hear that we are working together at least to try to coordinate what we're doing.

Okay. I've got the names, I have Allen, Gail, and Harlan Weisman down still after -- as soon as we come back from the public comment -- oh, you don't have a comment? Okay.

So, what we'll do now is stop for a minute and I need -- Sue will help us here with the public comment period. We'll first hear from people who are here in the audience. Sue told me we had two people and then we may have some people on the phone, but we're not sure about that.

So, why don't we start with the people who are here in the audience?

MS. SHERIDAN: Great. Thank you, Dr. Norquist. Gosh, this morning when we started out we had 13 people signed up for public comment and I'm afraid that we have gone down in numbers, but like Dr. Norquist said, we're going to take the public comment first --

CHAIRMAN NORQUIST: Let's hope that's because we answered all their questions.

MS. SHERIDAN: It could be, that's exactly what I think --

CHAIRMAN NORQUIST: -- it's not that they gave up on us. Right. Thank you.

MS. SHERIDAN: Right. So informative. But we'll start with the people first in our audience and then we'll go to those who are on the phone, and I hope some people have called back.

We will, for those of you who have written testimony, we ask that you submit that to PCORI.org and that all testimony that comes to us, we'll share either with the Board, the staff, or the

appropriate Methodology Committee members to address your comments or your questions.

So, first we'll start with Sara van Geertruyden from PIPC, who's going to share some comments, then I'm going to ask those of you who are offering to comment to limit it to three minutes please.

MS. VAN GEERTRUYDEN: Thank you. So, I'm going to read off my computer. I've been taking notes as you've been talking.

My name is Sara van Geertruyden, I'm the executive director of the Partnership to Improve Patient Care and today I'd like to focus my comments on three areas based on PIPC's prior work.

First, PIPC held a roundtable on May 8, 2014 with providers and patients of the Hepatitis B and C communities focusing on dissemination and implementation of clinical evidence, and I want to thank Jean Slutsky, she joined us at that roundtable discussion.

This roundtable was a spinoff of an earlier roundtable looking at dissemination more

broadly. The Hepatitis C community in particular felt strongly that having clinical treatment options available and new USPFTF recommendations for screening, their community was a great place to start in testing dissemination strategies.

But the summary and recommendations that we provided to PCORI clearly articulated the focus of conversation around screening, which has been and continues to be a serious impediment to treatment and an example of the challenge of implementing evidence in practice, particularly for hard to reach populations.

So, as PCORI pursues a research agenda related to hepatitis, we hope that this prior work is useful in your work to prioritize topics and we appreciate being invited to participate in your prioritization work in this area in the future as well.

Second, PIPC held a roundtable on June 19th related to accountability for patient engagement, so, better identifying how engagement is done in a manner that doesn't just engage the

patient, but activates the patient and their own care.

I want to thank PCORI for giving us a pointed response to our recommendation and we look forward to working with you in the future.

We provided several recommendations related to prioritization, conduct of research, and dissemination that could improve the quality of engagement and I'll describe just a few here.

Generally speaking, many of the recommendations focus on how PCORI can work more efficiently and effectively in its engagement by working with organizational stakeholders that can help PCORI reach those individual patients.

Organizations can help with things like topic solicitation and dissemination so that PCORI is reaching as many individual patients as possible. We suggested, among other things, that PCORI uses its ambassador program to engage organizations so that organizations on one side of the country can connect to organizations in another part of the country, and for dissemination

especially, it's not just about engaging patient organizations but also broad-based patient -- broad-based organizations that can help you target other particular populations, especially those that may be hard to reach populations.

I know in our conversations during the roundtable, organizations like Urban League would come up frequently in terms of people that may be effective for dissemination purposes.

There was also strong consensus in our group that there should be a patient voice on the Methodology Committee, both on the committee itself and in an advisory capacity.

The roundtable group saw your dissemination and implementation action plan as a key opportunity. Resources were allocated by Congress to do dissemination differently. The roundtable recommended that PCORI and AHRQ create advisory workgroups including patients, providers, health communications experts and researchers around the key areas of your research portfolio to those who advise on how best to disseminate

research, including where there needs to be capacity building in the community to get that information out effectively and what decision aids may be needed to effectively communicate evidence.

And I was happy to hear that AHRQ is also focused on decision support tools. I think that's a very key area.

We believe these recommendations support a process for dissemination that's consistent with the statute guidance, which is intended to support a more patient-centered dissemination process.

And third, PIPC is looking forward to publishing a white paper in the near future on the patient perspective for alternative payment models, which will look closely at how principles of patient-centeredness can and should be translated throughout the infrastructure of our healthcare system. PCORI and its patient-centered outcomes research is just the first pillar developing the evidence base that we hope will be translated into practice in a manner that both engages and empowers the patient.

It's important to recognize the power of your work in developing those best practices for patient-engagement as the broader health community will be looking at what you were doing with research to build the other pillars of a patient-centered health system, such as quality measures and the shared decision making tools.

So, in closing, I'd like to thank you for allowing PIPC to remain engaged and to provide input along the way. We're very excited about PCORI's work in the area of dissemination and to bring together stakeholders to provide -- to prioritize its research in a more strategic manner.

And if you want to look at any of the work that I've described here today, it's all available on our website at PIPCPatients.org.

Thank you.

CHAIRMAN NORQUIST: Thanks very much. Actually and thank you for the document that you sent. I know that we responded to that and they are very helpful. So that you very much. Sue.

MS. SHERIDAN: Thank you Sara. Wendy

Ellis, are you still here?

[No response.]

MS. SHERIDAN: I think that is no. So, I'm going to ask the Operator, Mike, do we have anybody on the line?

OPERATOR: No one is on the line at this time.

MS. SHERIDAN: Thank you.

CHAIRMAN NORQUIST: So, we would just remind people that we always take input at info@PCORI.org and also on our website, PCORI.org, so if you're listening and for some reason can't talk or something, that's another way to always give us input. So, we'll go back, unless someone comes up, we'll go back to our discussion.

Wait a minute. Did you want to say something at the public comment period or you want to -- oh, okay, go ahead.

MS. HOLE-MARSHALL: So, do you think that we could maybe build in a small public comment at each portion of the day, like one in the morning and one in the afternoon perhaps?

CHAIRMAN NORQUIST: Yeah, we could do -- that's a good idea.

MS. HOLE-MARSHALL: We could limit it, and then, you know, if there's too much spillover.

CHAIRMAN NORQUIST: Yeah, I think it's true, to make the public folks wait until the very end of the day, I mean, that's not -- I think that is not fair. I think that's a good point.

MS. HOLE-MARSHALL: Okay.

CHAIRMAN NORQUIST: So, we could split the difference. We have 30 minutes. We could split 15 and if we have to go over, we can, but let's try to put a 15-minute in the morning and then 15 in the afternoon, that way if somebody wants to come in the afternoon, they could come in the afternoon.

Plus, the other reason for the afternoon is for the West Coast folks who don't want to get up early in the morning, right? Thank you, Sue. Thank you, Leah. That's a very good point. They're up by now, I know, but I'm just saying --

[Laughter.]

CHAIRMAN NORQUIST: Okay, so, Allen, you

were up next.

DR. DOUMA: Yeah, I wanted to ask Rick a question. Rick, we're starting our Q&A back again just to let you know.

CHAIRMAN NORQUIST: For those who may be listening, we're back to the discussion we're having with AHRQ and our health system portfolio.

DR. DOUMA: This is Allen Douma. Rick, in the context of you guys running the National Guideline Clearinghouse and in the context of the fact that a lot of guidelines are not 100 percent evidence-based, how do you compare and contrast the dissemination of PCOR with the dissemination of guidelines?

MR. KRONICK: The National Guidelines Clearinghouse is certainly, you know, a piece of dissemination. It is dissemination primarily to kind of clinicians and not much, as far as I'm aware, to patients. And it's a fairly kind of high level -- you know, it's a very low touch. You know, the Guidelines Clearinghouse puts guidelines out there and if people can find them, fine.

I don't think, you know, it results in very much change in practice, although we don't have evidence on that, but I'm not sure I'm completely following your question.

DR. DOUMA: Well, one of the down sides, one of the questions we had a few years ago about whether we ought to actually develop guidelines, I think the resounding answer was no -- yeah, it was no, but a question is, if we want to be really patient-centered and be totally intellectually honest, should we be distributing information about how some guidelines are not evidence-based?

MR. KRONICK: Ah, I see your question. So, I mean, as you probably know, the Institute of Medicine, a couple years ago, I think 2011 -- I'm not sure of the exact date -- made a set of suggestions about tightening the criteria for inclusion in the National Guidelines Clearinghouse and we are in the process of implementing those recommendations so that, you know, guidelines that are in the Clearinghouse should have better standards of evidence than may have been the case

in the past.

You know, were they a sort of guaranty that every guideline that's going to be in the Clearinghouse is necessarily gold standard? I mean, no, probably not, and certainly there will be some guidelines that may even conflict with each other, but kind of this part of the job then is -- we don't see our job as being a final arbiter of exactly what's right, but we certainly are, you know, going through a process to try to make sure that at least some standards of evidence have been met before we put a guideline into the clearinghouse.

CHAIRMAN NORQUIST: Well, and I think certainly when they put the guidelines up they can say what methodology they used and then it's kind of buyer beware, but I do know that most -- because I know with my organization, my council that I'm on, is over them and the American Psychiatric, we're paying very close attention to what the IOM guidance is on this, but I think as long as you just publish what your methodology is then --

right? Okay, Joe.

DR. SELBY: So, even though I had heard these presentations or seen them before, I've still been really intrigued by the two and the similarities between the two as well as the differences and I also was struck by Sharon's call for big questions, and that one about how to restructure emergency departments within healthcare systems is a really big question and I think I would like to just ask the Board, and maybe especially Citizen Epstein in his last -- in the waning minutes of his public service on the PCORI Board, to weigh in on whether the distinction was clearly drawn between CER on systems and dissemination and implementation of PCOR and whether there are a class of really big questions that CER could address, like restructuring EDs and what did you have in mind way back then as we named improving health systems -- what did you imagine the kind of studies we'd be addressing with a CER lens?

DR. EPSTEIN: Yeah, so I think what Rick

has done and called dissemination was to change some of the words it could look very much like health services research for improving outcomes. Forgive me, Rick, I won't say it outside this room. I know it's dissemination, dissemination, but --

MR. KRONICK: We are at a public meeting, Artie.

CHAIRMAN NORQUIST: And it is being recorded.

DR. EPSTEIN: Exactly why I'm resigning.

[Off microphone discussion.]

DR. EPSTEIN: I'll stop there.

MS. HUNT: Go ahead.

DR. EPSTEIN: I just think -- so, an example that you could change a few variables, you could change some emphasis, and you'd be talking then about what are major health systems with integration now all the rage with more than half of physician practices no longer owned by physicians, with most hospitals now in multi-center chains, the kind of subject that he's trying to understand about, which is, how do these folks disseminate

effectively or how do they actually perform effectively, which is close, strikes me as a pretty big picture question. It doesn't disagree with Sharon's issue, but more than a one-off of, we tried three of these and two of those and it was a little more intense and worked a little bit.

He's also putting up fairly large dollars. I mean, he's got a big bet on this. I don't know whether it will pay.

MR. KRONICK: Joe, can I jump in? Because Arnie, what I heard Joe's question to be, and I wasn't here, clearly, when the original discussions occurred, but I gather that you were one of the supporters from listening to this, of putting 20 percent of the PCOR research effort into improving health systems PCOR, and I think what Joe was asking was, what were you imagining or thinking at the time that 20 percent should be funding.

DR. EPSTEIN: Yeah. I guess I was thinking of large trials, comparative effectiveness for the most part. There are some methodological reasons why they're a little better that way, that

tested important question about delivery systems that were not just everybody was asking this, but sort of a major question. Some of the ones we have unresolved, where are nurse practitioners and mid-levels and how do they really change the sorts of things we do?

And I had imagined that it would take lots of money, that it wouldn't be easy to do on $400,000 a year times three. But there'd be bigger questions to it.

We have other natural experiments we're doing now and CMMI is probably going to get the grail for doing it, but there are things like what happens when you bundle, what happens when you move integrated systems, what happens when you got ACOs, what's really worked for readmissions or not worked, how is it happening. Those are all big and they're about systems and I thought they might do as much to improve the healthcare and health status of Americans as Coumadin versus Heparin and I'm very sympathetic to Rick earlier today making the plea of we need more of those comparative -- we do

need that too. We need some lights on.

But that is what I was thinking. And I guess I just have the same reaction as Sharon when I was hearing the details, it just didn't seem like -- I gave it Harlan Krumholz's test, you know, I was pretending to tell a close relative which are the ones I was really excited about and I found myself stalled. I didn't have them.

For what it's worth, I like the one on falls. I think it's going to potentially tell us something.

I'm sorry if that's not more directive.

CHAIRMAN NORQUIST: Harlan? Oh, I'm sorry, you want to answer?

DR. LEVINE: Steve, first of all, I know you inherited this portfolio, you did not create it, but my -- what I meant to say and failed to say is, it wasn't about that those questions weren't questions that were worth answering, but they belonged in another one of the portfolios, some of them belong in the comparing clinical interventions portfolio rather -- to me, rather than improving

health systems because of the scale, that many of them or some of them, at least, are looking at different clinical interventions to achieve a better clinical outcome.

I wasn't trying to deride the question, but just in terms of where in the portfolio is the focus.

CHAIRMAN NORQUIST: Okay. Harlan?

DR. WEISMAN: Okay. So, one little point nobody probably cares about is, after hearing it a number of times and Gail whispering in my ear, I finally understood what Sharon was saying and everyone else in the room did and I now appreciate it and agree.

Okay. So, the question, Rick, you know, everyone has said, well, we better make sure that we're not overlapping too much, or at least intentionally overlapping and it makes sense, but I love the work you're doing and it's not -- dissemination with a small 'd', meaning, just telling and putting information out there, you've done -- AHRQ has done an impressive amount of it,

but Dissemination with a big 'D', meaning it has an impact that you want it to have, in other words, you've changed the way things are being done, it's incredibly disappointing, not an issue just for AHRQ but for healthcare in general.

And when I think about when I've seen effective changes, it's really when behavioral economic techniques have been used. And what I mean by behavioral economics is in a simplistic way, I mean that most of us overestimate the value of the present and underestimate the value of the future, so that we will gladly do something that gives us immediate satisfaction that later we're going to regret because it comes to cause harm to us, whether it's smoking, taking drugs, drinking too much, eating too much, whatever.

And what behavioral economics does is acknowledge that and adds something to the equation, which provides some immediate recognition of value, and sometimes it's just a simple reward.

You know, at Johnson & Johnson we had a very good record, still a long ways to go, but at

least when I was there, of changing employee behaviors by sometimes-trivial incentives or small monetary incentives towards better management of hypertension and diabetes and other things. Some healthcare systems have changed physician behaviors by merely giving them information about how they are performing relative to their peers, and that turns out to be a value. They don't like it if they're in the 2nd percentile among physicians who have diabetics under control.

And in world health studies, massive public health efforts have effectively used these behavioral economic principles to change behaviors at national levels. I'm just curious, have you -- has AHRQ looked at that or should we look at that? Because it seems highly effective.

MR. KRONICK: I think -- we have done some work on that and certainly we've done lots of work, as others have, on sort of figuring out how to get the incentives right and the -- behavioral economics is one piece of getting incentives right. You know, I am struck, surely, that at least in

looking at cardiovascular risk factors, while the evidence is not great, there's pretty clear evidence, I think, that there are some organizations, some healthcare systems that have done a much better job than others and we are, in both of these projects, actually, trying to sort of figure out how to make progress, you know, one focusing on the small- and medium-sized practices the other looking across health systems understanding what's been done, and I'm sure that, you know, that behavioral economics or explanations will be part of that, but I'm sure we'll also see, as we look across systems, big differences in choices that have been made about the workforce, mix of primary care and specialist, use of mid-levels, that -- I'm not sure we'll understand particularly well where those choices came from, but that's kind of part of, you know, what we need to figure out how to change.

DR. WEISMAN: Well, another part of that that you just alluded to, I think, was the notion of cognitive overload. When you have clinicians

seeing 30 or more patients a day, they can't possibly -- they are overloaded to a point that they can't possibly --

MR. KRONICK: Yeah, and the levers that -- I mean, the sort of main lever that the federal government and, you know, sort of private payers have is what they -- how they offer payment, which is a pretty blunt lever, and we are trying to develop evidence underneath that for clinicians and patients, health systems that are trying to respond to that, well, what can you actually do to improve. There's still, you know, the kind of fundamental question of how do you, you know, what are either the incentives or accountability mechanisms that will actually get people to improve, and, you know, depending on what you're trying to change, many different answers.

I think particularly, you know, if we're trying to sort of go back to the corticosteroids, and that's only one study, and more work is needed, but if it turns out that that holds up, well, that's a kind of big problem, or you have some

patient that goes into an orthopod and says, I'm in a lot of pain, what can you do, and it's not a great answer to say nothing, a much better answer to say, oh, here I have this shot, plus it may actually make some money for some people, so that's a sort of different kind of problem than the problem that we're facing on increasing blood pressure and cholesterol control. Each of these problems are going to have solutions that, as you point out, you need to pay attention. How do you get people to change?

CHAIRMAN NORQUIST: Yeah, isn't human behavior interesting? It's what I deal with every day. So, you know, there are all these different factors that have an impact on why people do things and they're not always obvious.

Alicia.

DR. FERNANDEZ: I agree with you. Thank you both for very interesting presentations. I want to be sure I understand, Rick, fully, the implications of something that you said and it has to do -- back to the corticosteroid example and if

I understood you correctly, AHRQ is not going to be doing these types of research -- producing -- funding this type of research in the future, sees that more as in PCORI's bailiwick, and I just wanted to make sure I understood that correctly and get a little bit of a handle, having sat in at an AHRQ study section and currently an NIH study section.

To me, this doesn't seem so much like an NIH question, I'd love to hear a contrary opinion, but when we talked about targeted PFA versus investigator-initiated, we had a pretty broad consensus of moving some more toward targeted PFAs, and yet somehow -- which I share -- but yet somehow, I can think of more good questions along the lines of the corticosteroid than I can think of good questions that are resolvable in our framework in the short time period with the amounts of money in a targeted PFA.

So, I want to invite you just to say a little bit about where you see studies of this type really fitting in between NIH and AHRQ and PCORI

and could you add to that a little bit just the order of magnitude of funding of studies, for example, of this one -- of this type? And then where you would personally see the balance between those two?

MR. KRONICK: So, I'll leave it to Mike, I'm not going to speak for NIH about what NIH sees as being in their bailiwick. Very explicitly, I will sort of confirm your question, that funding a study like the corticosteroid study or a study that will be coming out soon comparing ultrasound and CT for patients with suspected kidney stones is not work that AHRQ will be funding and to me it makes sense that PCORI should be.

Jean is trying to jump in --

MS. SLUTSKY: [Off microphone.]

MR. KRONICK: Yeah, and Mike, I don't know if you want to -- I don't want to put you on the spot, but whether you want to say anything about whether this is NIH's work. To me, it looks like PCORI -- it should be PCORI's work.

DR. LAUER: Well, I think, to some extent,

it is NIH's work and part of what we're doing right now, for example, through the collaboratory as well as through some of the individual RFAs that the institutes are putting out, is helping to develop approaches to conduct pragmatic trials in efficient ways.

But what we're really looking forward to, and this is something we've talked about a lot, is a point where we can more regularly leverage platforms like PCORnet so that we could -- we, potentially, at NIH could fund very large numbers of pragmatic trials, important patient impact trials like the order of magnitude of what you just talked about and be able to do that by virtue of the fact that PCORnet exists and that PCORnet is driving and functioning.

Right now if we wanted to -- I'll give you as an example, we're funding a pragmatic trial comparing an initial strategy of CT angiography versus strep testing in patients with suspected coronary disease. It's a 10,000-patient trial. It's going very well.

The results will be reported out I think sometime next year. This trial costs about $35 to 40 million. Imagine if we could fund a trial like that for $5 million by piggybacking it off of PCORnet structures. We could do so much more.

CHAIRMAN NORQUIST: Thanks. And Bob Zwolak.

DR. ZWOLAK: Zwolak, Board. Rick, Steve, thanks for those presentations. They certainly helped me. As I think of us moving forward and potentially reshaping the major buckets in which we put our announcements and our money, I wonder if you two would speculate just for a minute about the IHS, which seems to me to be pretty distinct and potentially different or separate from some of the other clinical CER trials.

When it comes to IHS, should we be looking at larger dollar trials, targeted trials, just broad announcements? In your perspectives, what's worked, what hasn't worked, and when it comes to IHS, where should we focus going forward?

DR. LAUER: Well, one thing we've been

concerned about is the ability of some of the smaller types of studies that have come out of the broad funding -- the traditional broad funding announcement really having the bandwidth to really be significant and particular issues of being able to do multi-site trials so that we can look at a diversity of clinical op settings that these trials could potentially be implemented in as well as having large samples so we could really look at some of the subpopulation issues within these types of system interventions in terms of what the real implications are for these different studies.

And so, for example, we've moved in the broad announcement towards this notion of a large broad option to try to get up to $5 million in five-year studies to try to accommodate some of those types of studies, which will enable us to potentially have that kind of bandwidth and the other area that I think is important is that with many of these complex, multi-intervention studies, which are being proposed in some of these areas, the ability to be able to have arms where we can

look at some of these options and really try to understand possibly what's driving it to see if these complex interventions are really the answer or are there elements of those interventions that could be considered appropriate.

Those do take larger studies to do that, in my opinion.

DR. ZWOLAK: And as a quick follow on, is $5 million enough? It seems like it's potentially not.

DR. LAUER: Well, I think we're looking at some of the -- it'll be interesting to see what comes out of the pragmatic studies initiative where that kind of doubles it to about $10 million and see what sorts of reviews and options we get from that particular -- my sense is that we are looking potentially to try to move to larger studies in order to make those kinds of potential impacts where different -- and in such a diverse healthcare system to be able to look at the diversity of those types of impacts will take, for some research questions, larger studies.

MR. KRONICK: I would agree with Steve that probably larger studies are often needed, but I would ask more fundamentally, and this is sort of backtracking -- I think what Sharon was raising earlier about whether these studies are studies that PCORI should be supporting. I mean, we had a discussion earlier this morning about kind of the need to focus and I think the question, for any study, is -- you know, has been raised a number of times, is what does the abstract look like when it's done and what's the -- what do we say to the Washington Post reporter, and then, more importantly, how is practice going to change and how are outcomes going to improve for patients as a result of this study being done.

And I think for many of these studies, it's hard to see the positive answer to that. You know, not for all, maybe but I think, you know, much easier to see that if PCORI's funding -- you know, doing -- injections of corticosteroids work or not, you know, in part it's a question for Steve Lipstein and others kind of running health systems,

but it's sort of what sort of evidence and information and they likely to take advantage of that PCORI might be producing. You might say, yes, fine, but I'm a little skeptical actually.

CHAIRMAN NORQUIST: Thanks. Harlan.

DR. KRUMHOLZ: Just since we're all around this table and I missed this morning, I just want to reinforce that point. I think it's so important. Arnie, you might want to be the one that speaks to this side, but to me, we're still not investing enough in the discrete trials that have to do with symptom relief, function, the kind of experiences the patients have, whether it's pain, things like incontinence, it's fatigue, shortness of breath.

I mean, when I see patients who come in who have a variety of depression, when people come in with these kind of complaints, depression aside, but a lot of the physical, somatic illness issues -- insomnia, I mean, we are grasping to try to figure out how to personalize the recommendations for them in a way that are meaningful.

You take incontinence, you know, someone will say, well, they should do Kegel exercise. Well, how many? When? What's effective?

I mean, what really makes a difference for people? I mean, no one's going to take the -- I mean, just -- so, I don't know, for $20,000 I think you could figure that out. I mean, just no one's directing at it, it doesn't become a priority, but if you really pull --

[Off microphone discussion.]

DR. KRUMHOLZ: Give me $20,000, I'll do it.

[Laughter.]

DR. KRUMHOLZ: I need a PPRN of incontinent people, but -- I know it's the end of the day, but my larger point is that I think these are the cycle -- if we can produce a big number of these where people are spending money pursuing different strategies, desperate at all levels of socioeconomic status across the entire country, are desperate for answers about how to get relief, looking at alternative and complementary

approaches, I mean, where is it that we're cycling through this kind of information that when you're on the wards or when you're in the office, you're hearing every day and you're just shrugging your shoulders like, I just don't know what to tell you because, you know, I'm sort of not sure.

If somebody told me their 90-year-old mom was going in for epidural injections, well, she can know not to get corticosteroids, but the question is, in 90 year olds, I mean, where are we organizing the data so that we can tell people, let's randomize and figure out how you feel and do the standardize collection.

I think that's the great need. Lots of money is being spent on a whole variety of either imaging or interventions that we don't know whether they work and that people are really, truly desperate to know what strategies will help me function better, be less symptomatic, and live a fuller life.

And to me, this is, I don't want to say cleaner or not, but the health system stuff is

really complicated and especially spreading it out and the terrain is changing all the time. That's what Steve's saying, he's absolutely right. I mean, next week it's different than it was last week, it's changing rapidly. It doesn't mean we shouldn't study it, but you're doing it, you're jumping in, and I think for us a proposed focus is on these things because the outcomes come fast enough that we can cycle the studies through fast enough and I'll say it one more time, if we can get the questions, we can hire the people to do the work, we can have it on time, on budget, and we can give away the data when we're done, de-identify it and give away the data because we'll do it, we'll let the academics present it, they can publish it with the patients and then the data will be owned by PCORI and then give it out and leverage it.

But I really think it's time for us to seize that moment and also, you know, recognize what AHRQ's doing. I mean, AHRQ's making -- it's a big investment you're making across the primary care and across U19 and I think we need to be

thinking hard about where our -- when we've got investments that we have to manage that we've already made, so the question is, how many more versus everything else that we need to do.

CHAIRMAN NORQUIST: Right, so that was the discussion, so we did that exactly in the direction in which we want to move because we only have so much money, we have only so much time, and we have to decide what are our priorities in all of this here, so we absolutely -- Allen?

DR. DOUMA: I just wanted to really reinforce what Harlan's talking about. If we're patient-centered, over the course of ten years, I or my company read two million emails from patients and I assure you they're more concerned about dealing with symptomology than anything else and we don't really take that head on.

CHAIRMAN NORQUIST: Okay, so Alicia, do you want to --

DR. FERNANDEZ: I wanted to jump in here because I don't -- I think we may be doing a disservice to some of the items in the portfolio,

which is not to say that I'm not on board with the -- that the discussion needs to happen, but for example, there are a lot of Patient Navigator proposals in there, and you could say, oh, well, Patient Navigator, I mean, like, who cares? Right? Well, all of my patients care, right, a lot of your patients care, right, their problem is they can't figure it out. The health system doesn't work for them.

So, they're not going to get in there to ask me about their insomnia and their incontinence and whatever because they can't maneuver the system as we know, it may not work well enough. When they get a bad disease, it doesn't work well enough.

So, I don't want to -- as someone who does health services research, and I sit on the NIH health services study section, I want to be careful that we don't throw out the baby in the bathwater because there are questions here that, in my opinion, will not get funded by NIH, are not going to get funded by AHRQ and are important to answer and that they are not the most -- I see the study

headlines very clearly. Patient Navigator helps low income patient achieve access, improve their outcomes, improve the treatment of their cardiovascular risk factors. That's the headline. That's not a bad headline.

CHAIRMAN NORQUIST: So, we're going to let Arnie -- since this is Arnie's last meeting, Arnie gets to kind of have the last word because he was raising his hand over here.

So, Arnie, we're going to put you on the spot here.

[Laughter.]

DR. EPSTEIN: That would be the wisest thing I could do. I don't -- this one's on, right, speaking to America? I could be Walter Cronkite.

So, I will just hearken back to some other wise words that Harlan Krumholz said, which really had to do with urging us to write the abstracts for studies that we funded and I think behind his request was the notion that if you could write the abstract, you could get a sense then about really how important this was likely to be, not a perfect

sense, but a pretty good sense, and I would submit, as I pass the baton for Improving Health Systems to Sharon and on, that that's really what's going to separate out PCORI. It's not going to be the 20 percent versus 40 percent or the 19 versus the 41, it's going to be, did we choose studies that were really important, that really changed what people did, and those people can be the doc on the street or the patient on the corner or the system leader and manager. So, I would focus there. And I think we could do that more effectively, even for the -- I know we've tried really hard and it's not easy.

Anyway, on that happy note, I'm happy to move for adjournment.

CHAIRMAN NORQUIST: All right. So, thank you, and let me just say, for all those on -- since we're over about 15 minutes here, that all of the information, the recording and all will be up on our website at PCORI.org and we're always happy, as I said before, to receive input at info@PCORI.org and at our website, PCORI.org.

So, we thank all of you for joining us --

well, if you make it quick, Joe.

DR. SELBY: I just learned that there's not going to be a microphone where we convene tonight, so I simply wanted to take the occasion of the fourth anniversary of PCORI and of your existence as a Board to thank all of you for your unwavering support and willingness to consult and provide consultation, advice, support, your participation and your interest in re-upping, as reported to me by the GAO.

I do understand that we'll have news about a new Board member to replace Citizen Epstein and all of those whose first term comes to a conclusion. We'll have news about all of that by the end of this month.

But we are appreciative. Sometimes I can't quite imagine what makes a person stay on a Board that requires so much of them as this Board does, but thank you all for staying on, hanging in there and supporting us.

The last thing I want to say is, thanks to Steve -- second last thing I want to say is thanks

to Steve for spearheading this governance transition work. I think it has worked remarkably well and the three committees are each feeling their oats now, I think, and really each have a larger bundle of work to do and that works really well.

And, in case anybody's wondering, Gray has done a remarkable job of stepping in for Gene and from my point of view, he's fantastic. So, thanks all. Happy anniversary. And, Gray --

CHAIRMAN NORQUIST: I know, by the way --

DR. SELBY: Gray, I know you want to get out of here, but just -- everybody should be aware that at last November's Board meeting I had to wear a Boston Red Sox hat to this Board meeting because I lost a bet to Rick Kuntz --

CHAIRMAN NORQUIST: Are you about to make a bet now?

DR. SELBY: I just want you to know, I can't possibly have to wear that hat again this year. It'd be Oakland As this year.

CHAIRMAN NORQUIST: So, we meet at 6:30 in

the lobby for those who are going. By the way, there is a fourth committee, FAC, and we want to thank you guys for all the hard work and stuff that you've done.

[Whereupon, at 6:00 p.m., the telephonic portion of the PCORI Board of Governors meeting was concluded.]