PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

BOARD OF GOVERNORS MEETING

Monday,
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9:04 a.m.

The Park Hyatt Hotel
1201 24th Street N.W.
Washington, DC 20037

[Transcribed from PCORI teleconference.]

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APPEARANCES:

BOARD OF GOVERNORS

Kara Ayers, PhD (via telephone)
Lawrence Becker
Francis S. Collins, MD, PhD
Jennifer DeVoe, MD, DPhil
Alicia Fernandez, MD
Christopher Friese, PhD, RN, AOCN, FAAN
Christine Goertz, DC, PhD (Chairperson)
Michael Herndon, DO
Russell Howerton, MD
Gail Hunt
Sharon Levine, MD (Vice Chairperson)
Freda Lewis-Hall, MD
Michelle McMurry-Heath, MD, PhD
Barbara J. McNeil, MD, PhD (via telephone)
David Myers, MD (alternate for Gopal Khanna, MBA)
Ellen Sigal, PhD
Kathleen Troeger, MPH
Janet Woodcock, MD
Robert Zwolak, MD, PhD

AGENDA

AGENDA [continued]

P R O C E E D I N G S

[9:04 a.m.]

MS. JACKSTADT: Dr. Goertz the floor is yours.

* CHAIRPERSON GOERTZ: Thank you Kat. Good morning everyone and welcome to the December 9th meeting of the PCORI Board of Governors. I'm Christine Goertz, the Chairperson. I want to welcome those of you who are joining us for today's Board meeting, which is being held in Washington, D.C. and by teleconference and webinar. Thank you to everyone who's joined us in person, online, and on the phone. We're very pleased to have you here.

All Board members are present either in-person or on the phone with the exception of Gray Norquist or plan to be present for the meeting. I want to remind everyone that disclosures of conflicts of interest of Board members are publicly available on PCORI's website and are required to be updated annually. In fact, you've been recently sent a reminder to update those disclosures so if you haven't done so already, please make sure that

you do both before January 31st, 2020.

Board members are also reminded to update your conflict of interest disclosures when that information changes. If the Board today will deliberate or take action on a matter that presents a conflict of interest for you, please inform me so we can discuss how to address the issue. If you have any questions about conflicts of interest disclosures or recusals relating to you or others, please contact your staff representative. All materials presented to the Board for consideration today will be available during the webinar and then after the webinar will be posted at our website, www.PCORI.org. The webinar is being recorded and the archive will be posted within a week or so.

We have a scheduled public comment period today from 12:30 to 1:00 Eastern time. If you're interested in registering to provide public comment, please visit our event page for instructions. Alternatively, you can always email us at info at PCORI.org or provide input through our website at www.PCORI.org.

Finally, a reminder that we're live Tweeting today's activities on Twitter. Join the conversation @PCORI -- with @PCORI.

In just a minute I'm going to turn the mic over to our new Acting Executive Director, Dr. Josie Briggs but before we start a couple of -- I want to make a couple of comments. First, I wanted to let the Board know that Trent Haywood has resigned from the Board. He will no longer be representing in the payer sector and as a consequence has decided to resign. Obviously we'll miss Trent and appreciate his work while he was a member of the Board and wish him well in his future endeavors.

I also just want, want to comment on the fact that it feels very different at the head of the table at this meeting than it has in previous meetings for many reasons. Gray is now in Paris while we're here and I'm sure he is missing us right this minute and wishing he was here and instead, but you know, just one more heartfelt thanks to him for the excellent, his excellent work as chair for the last six years. And then obviously, the fact that

that Joe is not sitting here at the head of the table, it's something also that it's definitely a sign of change. And Joe, I imagine you're sitting somewhere behind me. I can see you in the mirror. I just want to thank you for your tremendous work as our founding Executive Director. We are -- PCORI is forever grateful for your dedication and your commitment to our shared efforts.

And now Josie I'd like to turn it over to you.

UNIDENTIFIED SPEAKER: We're going to do a roll call.

CHAIRPERSON GOERTZ: Okay. Thank you.

MS. JACKSTADT: If you could please indicate your attendance by saying here. Cara Ayers.

DR. AYERS: Here.

MS. JACKSTADT: Larry Becker.

MR. BECKER: Here.

MS. JACKSTADT: Francis Collins. Jennifer Devoe

DR. DeVOE: Here.

MS. JACKSTADT: Alicia Fernandez

DR. FRIESE: Here.

MS. JACKSTADT: Christopher Friese. Christine Goertz.

CHAIRPERSON GOERTZ: Present.

MS. JACKSTADT: Mike Herndon.

MR. HERNDON: Present.

MS. JACKSTADT: Russell Howerton. Gail Hunt.

MS. HUNT: Present.

MS. JACKSTADT: David Myers, filling in for Gopal Khanna.

DR. MYERS: Here.

MS. JACKSTADT: Sharon Levine.

DR. LEVINE: Here.

MS. JACKSTADT: Freda Lewis-Hall. Michelle McMurry-Heath. Barbara McNeil.

DR. McNEIL: Here.

MS. JACKSTADT: Gray Norquist. Ellen Sigal.

DR. SIGAL: Here.

MS. JACKSTADT: Kathleen Troeger.

MS. TROEGER: Here.

MS. JACKSTADT: Janet Woodcock. And Robert Zwolak.

DR. ZWOLAK: Here.

MS. JACKSTADT: Terrific. Thank you Dr. Goertz.

CHAIRPERSON GOERTZ: Thank you, Kat. Josie.

DR. BRIGGS: Thank you very much Christine. Can we bring up my slides please? Next.

Oh, do we need to vote on minutes?

CHAIRPERSON GOERTZ: We're not quite there yet. This is just if you have any casual opening comments.

DR. BRIGGS: Okay, I see. So backing up just -- thank you Christine.

We have a packed day planned with I think a very lively and interesting agenda. After the minutes for you will hear my comments about where we're going and the Dashboard report and then a presentation from Kristin Carman about patient-centeredness and engagement and then a presentation

from Neeraj Arora about our cancer portfolio. We think you're going to find this a very interesting morning.

Back to you, Christina.

* CHAIRPERSON GOERTZ: Great. Thank you. Thank you. So the, the first order of business is approval of the minutes. So I'm going to ask if there are any additions or corrections to the minutes.

DR. ZWOLAK: I believe in a table that describes improving methods for conducting PCOR there's a typo because it currently says new causal interference methods.

[Laughter.]

CHAIRPERSON GOERTZ: All right.

DR. ZWOLAK: And that should be new causal inference methods.

CHAIRPERSON GOERTZ: So noted. We will we will make that -- make sure that correction is made.

All right. So pending that correction, can I have a motion and a second to approve the minutes? Bob since you're -- thank you.

MR. BECKER: Second.

CHAIRPERSON GOERTZ: So we got Bob Zwolak as a first and Larry Becker as a second. Any further discussion?

[No response.]

CHAIRPERSON GOERTZ: All in, all in favor?

[Ayes.]

CHAIRPERSON GOERTZ: Opposed?

[No response.]

CHAIRPERSON GOERTZ: Abstentions?

[No response.]

CHAIRPERSON GOERTZ: Great. Thank you.

All right, so now I will introduce, again, Josie to deliver her opening remarks.

* DR. BRIGGS: Well, I'm really delighted to be with you all today. As you know, I've been engaged in a six week crash course in learning about PCORI and I suppose my first thing to say is what an extraordinary achievement of it has been over the last six to eight years, led by Joe and so actively designed by a very engaged board, some of whom are still members today. I think the achievements have

been spectacular and I am thrilled to be -- to take on the complex task of being an interim as PCORI prepares for PCORI 2.0.

I will add however, I say that it's about bringing patients into the discussion. There is no time for complacency. The need for the kind of research that PCORI is doing is greater now than it ever was. We have a healthcare system facing many, many challenges and the kind of evidence that the PCORI's charged with building is a strong and persistent need. We will over the next day and a half think together about how to prepare the organization for PCORI 2.0 and a new Executive Director building -- who will, we hope, follow in the incredible tradition that Joe set as the leader of this organization.

But PCORI is also truly blessed by a strong and engaged board. I, as your Interim Executive Director, report to you, I'm answerable to you. I have benefited over the last six weeks with one-on-one discussions with some of you, not with all, but certainly with conversations regularly with

Christine and Sharon.

But this is this next day and a half is my first opportunity to meet with -- face-to-face with most of you. And so, I am really hoping I will get from you guidance on what are the priorities for an interim executive director. I believe an interim period can be a great value to an organization and to the new permanent director, allowing careful consideration of strengths and trouble spots, refinements of processes, clarification of sub-governance and so on. Most critical decisions about operations will, of course, we left the new leader, but I hope to help prepare the way and what to make interim period as useful to the organization as I possibly can.

So this is an open invitation to all of you to help me in this important priority setting for this complex transition phase.

What I'm going to cover today is first of all the indicated Dashboard summary that summarizes the metrics for achievement over 2019 and I think you'll find that information very interesting. And

then I'm going to delve into some depth into a completed PCORI-funded study from results to impact. And I think that story illustrates what is I think an emerging PCORI vision. And I think this will also provide meat for further discussions.

So here's where we are in the quarterly calendar of the Dashboard. It is according to your processes, a requirement that the executive director come back to you at this point at the end of the fiscal year, which ended September and report on a set of metrics that you have established to see how the work is going. And out of this conversation I think you will find the metrics and the things I'm going to tell you about highly informative. But keep in mind, this should also be part of a process to continually improve those metrics so that we have ways to measure and quantify PCORI's work.

So this is the slide and this shows all the metrics that the Board governors have developed. Only one is in the yellow zone, and that's in funds committed due to the fact that somewhat fewer meritorious applications were received in the summer

review cycles. The funds committed in quarter four were less than budgeted.

However, the applications you approved in November, where we had a robust slate, will largely correct this imbalance. And just as referenced to this, this is the funds committed -- cumulative funds committed through the end of Fiscal Year ‘19 and budgeted for through the end of Fiscal Year ‘20. I think it's useful to place these numbers in context. So I just wanted to remind you of the funding commitments that cover through the fiscal year that just ended.

Seventy-nine percent of PCORI’s funding commitments have been to research projects, 1.9 billion. A smaller commitment, 15 percent has gone into research infrastructure and substantially more modest commitments to engagement in research and research on dissemination and implementation four percent and two percent respectfully -- respectively.

The next metric I'm going to talk in some about in some greater depth is research project

performance.

These are some numbers that clarify PCORI's results to-date on certain metrics for research project performance. I gather the Board at a recent meeting talked some about the problems in recruitment. Anyone who's been involved in overseeing clinical research knows that recruitment for projects is always challenging. PCORI currently to-date shows 64 percent of the original enrollment target was achieved. That compares, and there's several references there, to other funders where the number that emerges is 55 percent. Sixty percent achieved that recruitment target within the agreed upon recruitment target timeline and a certain percent, 37 percent, require an extension of the recruitment target.

These numbers are very comparable to numbers that are quite familiar to me from overseeing similar kinds of work at the NIH. Six percent of projects are not successfully completed. I'm actually quite impressed with PCORI's processes for monitoring project completion. They compare

favorably with the best processes I've seen at the NIH and these numbers do not surprise me at all.

It is, however, of course, a glass half-full. It is hard to get clinical research successfully completed and the problem of achieving broader buy-in and indeed better process to get these important studies done as rapidly and effectively is important.

The next metric I want to talk about is timeliness of getting results published. Again, this shows a benchmark 60 percent at 60 months and shows the line for PCORI projects to-date. Again, a half-full/half-empty kind of situation. Most projects at 20 percent have not published at 12 months and or more than 20 percent, but by the 30-month mark PCORI has achieved the benchmark seen in other parts by other funders.

This should however, be compared with an important, innovative process that PCORI has established, which is our own internal peer-review process. And this shows the timeline for PCORI results availability through the internal peer-

review process PCORI has established. This is highly innovative business. I do not know another funder that is as effectively moving results into publicly available reports as this. It's challenging. It's involves very careful scientific review.

It is led by Harold Sox who's very experienced former JAMA editor and I think they're doing a superb job. They do occasionally encounter -- so in general we are encouraging authors to submit -- investigators to submit through this peer-review process before the recovery results will be available online. And generally that's happening, although occasionally there have been some controversies around journal embargo policies. But this is, I think, a very important step toward progress.

And it's important because of the susceptibility. Anyone who's done systematic reviews knows that there's an inherent worry about publication bias. Do the most positive findings get published and the others don't? We really want to

have safeguards against that and I think PCORI is very innovative peer-review process is doing a good job at that.

The next metrics I want to talk about are uptake into UpToDate and other examples of uptake into policy and systematic reviews. I carry, like most physicians, UpToDate on my cell phone and I use it all the time. I used to tell people that the way to judge a research finding, is do they have to rewrite the textbooks. Now I would say do they have to rewrite UpToDate. It's for a lot of the kind of work that we do, an interesting and important metrics.

Just an aside here, about half our projects are in the kind of completed phase. That's that bar of 291 and about half are in earlier stages or ongoing. So those numbers are worth keeping in your mind as you look at the actual numbers for what's happening. So these are the uptake into UpToDate which is shown at the left. That's -- so the first year this was tracked, four projects were incorporated into UpToDate in ‘18, 12 and last year

20 projects became -- resulted in change of UpToDate or were cited in UpToDate and may have resulted in some changes, recommendations. And you also see the numbers for citations in other evidence-based clinical recommendations uptake into systematic reviews, uptake into policy documents.

I view these as impressive metrics for impact.

Many of you are familiar with use of citations as a measure of impact. I edited a journal and I'm very familiar with issues around citations. Citations are a lagging metric and they do not necessarily tell us, although they are important. Citations, I think, do not tell us on the likelihood of a result being on a step towards implementation the way these metrics are.

So I think these are impressive findings and tell us that PCORI work, even the half or so that are done are really having an impact on our health care -- thinking about healthcare issues. And I remind you this is only about half of the projects that are ready for this kind of a step

forward.

Now we will talk and continue to talk, I hope, about implementation and our portfolio of projects ready for dissemination and implementation awards is growing. And these numbers just show you the funds committed to dissemination and implementation awards in 2019. Still a modest component of our overall funding. But I think an important one in that all these projects have been judged ready for further dissemination and implementation. I think this is an issue we should all talk about more together.

And then one last area that I want to talk about the metric is new studies using PCORnet. About two months ago, NIH announced a $90 million study. Remember, that number -- it's an important number that will utilize PCORnet.

This is a study called PREVENTABLE. It will test whether statins prevent dementia and other cardiovascular disabilities in older adults who do not currently have an indication for use of this class of drugs. It's a control. It's a placebo.

It is using a generic statin drug. This study will recruit approximately 20,000 study participants and follow them for four to five years. Fifteen thousand of these participants will be recruited through the PCORnet infrastructure and 5,000 through Veterans Administration hospitals. One of the impressive features of this study is that the cost per patient per year is about a thousand dollars. It's about a tenth of the typical costs of large randomized trials such as those typically run by the NIH and perhaps as much as a twentieth of large what industry trials often cost.

So I see this as an incredibly important step, proof of concept in the hope for PCORnet that it would help us learn how to do large randomized clinical trials more effectively and we will see, but perhaps even faster. Time will tell.

Why the huge savings? Well, one reason is that the infrastructure that exists through PCORnet and the data, the distributed data model, and the common data model enables the investigators to pre-identify 1.5 million patients potentially eligible

for enrollment in this study. So think of that. If you've ever run any large trials to start out with a list of many more patients than you need who would meet your enrollment criteria and PCORnet's capabilities to directly address both patients and providers to inform people and enroll people in this trial.

It's probably the single element that is most revolutionary in the infrastructure for this study.

But another element is that as part of its patient centeredness and I understand the planning of this study has truly been patient-centered. The investigative team has recognized that maintaining engagement in this study will be facilitated by much more use of home-based visits and much less reliance on medical center visits by people in the study. There will probably be one visit or two, but substantially more of the interactions will -- with subjects, will use electronic means and home visits to maintain and engage -- maintain the engagement of patients in this study. So I think -- we in

cooperation -- so the study is being funded jointly by the National Institutes of Aging and NHLBI. But I think we at PCORI can take real pride in the infrastructure that was developed with PCORI's investment to facilitate this interesting work.

One aside, this would not fall in PCORI's mandate. This is not a comparative effectiveness study. These subjects are not currently considered appropriate for statin prescriptions. So it isn't comparing two interventions or usual care versus an enhanced intervention. It's a placebo controlled study of a new indication for statins, but I think it's one that all of us over a certain age can be very interested in what will the results be.

So just quickly, here's the summary of some of the things that I've talked about. PCORI has to-date committed about $2 billion to fund 639 research studies. That's been through three types of solicitation. Targeted research funding, which is about $560 million. Pragmatic clinical studies, which is about another half billion and broad research funding, which is about a billion. To-

date, we have had 291 studies completed, ready for results to be posted in abstract form and 1,700 publications.

The other important categories, funding has been the $522 million that has gone into research. What we're calling here research support. That includes the $350 million that's gone to build PCORnet, $100 million, a much more modest sum, in engagement awards and a new program -- $41 million in dissemination and implementation projects.

And I've led you through some information on early uptake of research findings, including citations in UpToDate, citations in clinical guidelines, systematic reviews, and policy documents. I view these as extraordinary achievements for a young organization and I think the Board members should take great pride and indeed so should my predecessor and -- Joe Selby, this is I think truly a vision realized. In spite of the fact that the healthcare system still has lots of problems and there's a lot to be done.

So just to illustrate, I think an example

that I believe really illustrates what I see as a PCORI vision coming together.

I want to take you through a sequence from results to impact on one study that was published in JAMA about a year and a half ago. This study was led by Jeffrey Gerber and his team at CHOP. It compared broad and narrow spectrum antibiotics for children with ear, sinus, and throat infections. The results were published in JAMA. Result summaries were posted on PCORI.org. The final research report was posted in PCORI.org. It was incorporated into PCORI's evidence updates and then used by several external authorities for their own activity. And in November you approved the funding of an implementation project and I will also show you an economic estimate of its potential impact.

So this is the study. It was a large retrospective cohort study comparing the effectiveness of broad versus narrow spectrum antibiotic treatment for upper respiratory infections in children. They worked with patient families to identify and gather key data on patient-

centered outcomes and found that narrow spectrum antibiotics were associated with lower rates of adverse events, higher quality of life, and no difference between patient outcome in terms of the duration of the infection.

This shows the citation up there. In the right corner you see the Altmetric score 437. I'm a journal editor, so I know that's actually a pretty spectacular score. This JAMA paper attracted a lot of public interest.

PCORI then went ahead and prepared a result summary for the general public and a professional abstract. This was the most viewed results page on the PCORI website with 3,700 page views. The next step was preparation of that complete final research report. This is pretty detailed 93-page open-access write-up of more detail about the study than JAMA would allow in their page limits and provides interested investigators ready to keep going with this research issue, essentially all the critical details about the study.

And this is the process that Harold Sox

oversees to make sure that these are rigorous, carefully reviewed reports.

Based on that, we all know one study is not considered an evidence base. So PCORI also introduced, developed evidence updates for parents and for clinicians that incorporated these new findings with the existing evidence base. And this finding was quickly brought to further attention by external authorities; the Urgent Care Associations, Antibiotic Stewardship Council, incorporated the results into their resources and link directly to our summaries was also uptake into Wikipedia where it is cited on three Wikipedia pages. So these are important external evidence that this study is attracting a substantial interest.

All of you are aware of course, that although antibiotic stewardship is important for the individual patient, it is also important for all of us because of the growing problem of antibiotic resistance and is an impact of this study that extends beyond it's impact directly in the setting of these specific decisions.

An implementation project was submitted by the team from CHOP, by Gerber and this is the project that you all approved for funding at your November meeting. This will test strategies to improve antibiotic prescribing for more than 350,000 children across five health systems in three states. How to move findings from the literature, from guidelines into actual practices as everyone in this room knows is a very tough hurdle. And I think that this implementation science project will teach us things about doing that. So it's -- and we'll evaluate the actual impact on provider practice. So I see this as a very important and highly novel aspect of the PCORI's mission.

PCORI has also contracted with a group with expertise in and assessing the financial impact of this study and the estimates that were developed. This is available, this financial impact study is available on our website. It is estimated that it will have a substantial reduction in adverse drug reactions in emergency visits, in outpatient visits, and financial positive impact for Medicaid and for

other payers. This does not measure what the impact is on reducing the problem of antibiotic resistance, which is pretty hard to quantify, but is of course, an important long-term societal impact of this kind of work.

So that's an example to my mind of a true PCORI vision, of a project that is led through a series of steps that will I think move important research findings to changes in how healthcare is administered. And I thought that would be an illustrious example to initiate our discussions today on where PCORI is going next and to provide you with some food for thought as you advise me on what are important short term goals for an interim director and indeed how we can and the team behind me, a fabulous group of individuals, how we can help as you develop your vision for PCORI 2.0.

So thank you very much for your attention. I look forward to your comments.

CHAIRPERSON GOERTZ: Thank you Josie for that really excellent presentation. That was just an amazing way to kick off our day and, once again,

I want to thank you for taking on this short-term mission during this extremely critical period and PCORI's evolution. We're very grateful.

DR. BRIGGS: Thank you.

CHAIRPERSON GOERTZ: All right. Why don't we go ahead and open up for discussion. First of all, let's -- if anyone has any comments or questions regarding Josie's presentation. Larry. Bob, I'm assuming that your tent card is still up from -- thanks, Larry.

MR. BECKER: So one thing, putting my business hat on for a minute and going back to the slide 31 where you just put the economic impact.

I mean one other thing I would add to this slide is what our cost was to do these projects so that we see what the R01 is on this investment. So the world can see what we spent, you know, whatever the million dollar number is versus $131 million or the $118 million impact because I think that's important that, you know, we're not spending $118 million to get $118 million. We're spending a million or two to get that.

DR. BRIGGS: Thank you Larry.

CHAIRPERSON GOERTZ: Thank you. Mike.

MR. HERNDON: I just want to say this without beating a dead horse. Dissemination is really where we need to be focusing going forward. As a payer it's great to have the research but still when it’s seeing it and using it -- where's the bag? Appreciate your emphasis in your talk about that.

CHAIRPERSON GOERTZ: Thanks. Thanks Mike. Steve.

DR. GOODMAN: I thought the slide showing the publication, the reporting of results through our peer-review process versus those in the literature was really quite striking. I don't know that there's -- there's no funding agency in the world who has an alternative mechanism and it's absolutely right that most funding agencies are struggling to get in the, you know, 60 percent, 50 percent range within just a few years, no less at five. And we're getting 100 percent or very close to it in a very short time period.

I'm wondering, so that's something is a

model and something really to be trumpeted. I'm wondering if this is also an opportunity and maybe we've done it to explore because we have the reports in hand, why they're not -- why that 40 percent or 30 percent is not being published. I mean we're, we're engaging with the investigators. We know who they are. We have the reports in hand and there is no such thing as a non-publishable study once it's already been written up and gone through peer-review, there's literally no such thing. Maybe at two percent.

So I would very much want to know how hard that -- you know, whether they're submitting and if they're not submitting. Why?

CHAIRPERSON GOERTZ: Thank you.

DR. BRIGGS: Thank you Steve. I think this is an excellent point. And I think that findings that disappoint the investigator, they expected to see large, highly significant benefit and they don't see it is a common problem that I'm familiar with and those findings should be written up because we do not want the publication bias of effects that are

smaller than expected or maybe even going the wrong direction. Those are just as important.

So I do think this is worth more outreach and also perhaps a cite-by-cite attention too. It's a good point.

CHAIRPERSON GOERTZ: I think that if I only publish positive results, I'd only have about 12 publications. It's just incredibly, the negative studies are just as important to publish.

And speaking of publications. I agree that it's really important for PCORI to be evaluating impact and our -- what's called our hit rate, you know, in these broader terms and I also, I agree with Steve that that there's an opportunity here that we are at the forefront of thinking about things in some unique ways and our staff starting to think about how to publish some of these processes and the results because it seems like that would be one important short-term goal for an interim director to think about where are some opportunities to work with staff to get some of this out there.

DR. GOODMAN: I just want to add, I

actually doubt that it's publication bias on the base of the results, although we need to look at that. It's probably the studies that have not reached accrual targets and other reasons for failure, but we should really get a really good handle on that. And if they're not reaching accrual targets, we need to look back at the application phase and see why are they projecting so much higher accrual rates than they achieve. Very frequently they don't -- investigators don't actually look at the empirical evidence in their own institutions about the availability of patients.

DR. FERNANDEZ: It just needs a look at but I think it'll probably be some of both, but I think Steve is probably right because you know, obviously sample size calculations of the same, whether the trial is for a three-year trial or five-year trial or whatever. PCORI studies are short and that puts an enormous amount of pressure on the accrual within a short amount of time, all of which is to say that these results are even more impressive from the perspective of say, comparison to say a five-year

R01 on a trial.

But I think that at the end of the day, this is an empiric question. We can find out the answer to this and we should. What my takeaway take away from your outstanding presentation was we're actually PCORI is actually doing really well and that is a very heartening in terms of the comparison to particularly even when compared to institutions of the magnitude and most of the longevity, the maturity of the other U.S. research institutions such as the NIH.

CHAIRPERSON GOERTZ: Thank you, Bob.

DR. ZWOLAK: Josie, thank you. That was an outstanding presentation.

My question is that with a fresh set of eyes, how do you view the peer review process and the utility of our peer reviews there are done at two levels. They’re done at a lay and a scientific level. We spend substantial resources creating them. Are they adequately utilized? Is there -- it seems like there's an enormous amount of effort that goes into these and are we making the most of the

peer reviews?

DR. BRIGGS: Yeah. These are important questions. And I have done my own browsing of the Web to get some sense of the reports and the quality, but I have to admit in six weeks, I haven't delved into this issue with the depth that it deserves.

I am impressed out that we have a very knowledgeable team doing it. But I suspect there are ways we could improve the accessibility and impact of this effort and perhaps we can do it a little more efficiently. I don't know yet. It's a good question. I'll add it to my to-do list.

[Off microphone discussion.]

DR. BRIGGS: I don't mean that in a negative way.

DR. ZWOLAK: I'm sure your to-do list is very, very long. But it is, I think it's interesting that we could potentially evaluate that at some point.

CHAIRPERSON GOERTZ: All right. Thank you. Any other comments on Josie's presentation? All

right. Kara?

DR. AYERS: Yeah, I was interested in the dissemination to policymakers. So it came up 45 times, which I thought was pretty impressive and I didn't know if -- I'm assuming that that's all sorts of policy levels. It's in the slide with the -- do you know what I'm talking about?

More so a comment that I thought that that was a great marker for dissemination and that I hope that that continues. I don't know if that is somewhat reflected by our increased communication with policymakers related to the reauthorization efforts, but either way I think it's a great indicator and I hope it continues.

DR. BRIGGS: Yeah. You know, what? I think it's an very interesting metric and I will at some point have Michelle brief all of us on some of the specific examples. It is an important distinction that we make when we talk to legislative voices. Our job is to generate the evidence that's informative for policy. Ours is not the role to set policy. And this is a distinction that I sometimes

find the communities can find confusing, especially researchers, and I'm familiar with that tension at the NIH. But I do think it's an important distinction and understand how and to indeed have the goals that our science is relevant to policy.

I edit the leading nephrology journal and my -- I say the same thing to our authors. It is our job to publish the best and most definitive science related to policy and then hope that a thoughtful dialogue about policy ensues and I think it's an important part of our role here.

CHAIRPERSON GOERTZ: Thank you. So Josie has asked us to consider two questions. The first are some important short-term goals for an interim director. And certainly Sharon and I have had numerous conversations with her about what we hope that she will tackle. And that includes obviously keeping PCORI running in a very smooth manner during this time of transition and to -- but also to use her outside, you know, eyes and previous experiences to really do an informal assessment of ways that we may consider process improvements as we look at

PCORI 2.0. But we'd really appreciate hearing from Board members if there are additional suggestions or areas of emphasis to be triaged among the things that are already on Josie's list. Ellen.

DR. SIGAL: Well, I assume you spoke to all Board members about this because we had conversations on the short-term goals are to keep the ship running. Whether we need to reboot and really have to relook at our processes more extensively. I think is incredibly important. We have accomplished a lot and from where we started to where we are now, but we are relatively bureaucratic and not as fast or facile as we can or should be.

So how we do that and when we do that is a big question because I think that in all likelihood we will be re-funded and I think we have to kind of look at how long it takes us. What our processes are. We talked about peer review and other things, but I do that would be in order. When we do it or how we do it is another question.

CHAIRPERSON GOERTZ: Thank you. I could not agree more and beginning to again to identify

some of those some of those areas for further consideration as we look towards bringing on a permanent executive director. I think that the timing is really ideal for that.

Any other thoughts or -- Mike?

MR. HERNDON: I think helping us frame a more effective and meaningful topic selection process.

CHAIRPERSON GOERTZ: Thanks. Well, then I, you know -- Barbara, go ahead.

DR. McNEIL: You know, I would just second what Mike said. I think we would be a better agency if we had better topics in general. I know we've had several great ones, but I think we've had some on the lower end that we could have done without.

CHAIRPERSON GOERTZ: Thank you Barbara. Robin.

DR. NEWHOUSE: I would just add our continual effort to link the Methodology Committee members and their expertise to ways we can help advance the PCORI mission. We're always ready to be able to engage in those conversations and

committees, small task force, whatever we can do.

CHAIRPERSON GOERTZ: Thank you Robin. I just want to agree with that and as we look at the PCORI 2.0 to really figure out how we can better leverage the amazing expertise that we have on the Methodology Committee, I think needs to be a high priority.

All right. The, the next question that that Josie brought to us is how can PCORI staff help the Board prepare for planning for PCORI 2.0? And we've already begun that process with the conversations that Sharon and I have had with I think most of you and hopefully we'll be able to get those last conversations completed in the next -- before the end of the end of the year. And thank you all for, you know, these really helpful and forthright discussions.

I think it's been really helpful and we'll be talking about that a little bit more in the future as we start to get a little bit more specific about what the plan is for the PCORI 2.0. But any suggestions you have on things that you think would

be particularly helpful for, you know, background information, et cetera, that you think would be helpful for us to be thinking about just even in this in this preliminary phase, what would be helpful.

Ellen.

DR. SIGAL: So recently on a project that I'm working on at NIH, which I will not talk about, is we're looking at metrics for looking at timelines, how quickly and what our processes are and how quickly we get the information from drug approval to implementation and the clinical trial.

So I think some data would be really helpful about how long it takes us from the time we actually fund a project from when the first applications come in would be important. So I think some metrics and some data collection would be very helpful to inform us on our decisions.

DR. BRIGGS: Yeah, I think we could certainly refine some timeline-kind of metrics and add them and perhaps bring that back to this Board as to adding to the Dashboard metrics.

CHAIRPERSON GOERTZ: Thank you. Gail.

MS. HUNT: Following up on this mention of how we go about deciding which projects or which content are we going to focus on. My understanding is that, and I guess everybody's, is that some of the legislation that's being proposed, there are specific topics that Congress wants us to look at, but it's like four. So maybe it would be really important for the staff to help the Board understand beyond those, what are topics that would be really important for PCORI to be funding?

What areas have, I mean, you know, we've got the 100 that the IOM came up with and we brought them from different places, but what are maybe another four or another six that would be important in addition to whatever Congress is going to come up with for us to be funding. It would have impact.

CHAIRPERSON GOERTZ: Thank you Gail. I think that that is the really important question and something that we will definitely be talking about as we look at the PCORI 2.0, is how broad should our portfolio be during this next phase? It can be both

exciting and frustrating, too. I mean, right now our mandate is, you know, can conceivably cover, you know, all diseases and all populations. It's not possible to do all things well. And this is an opportunity for us to have, I think more -- we've talked about focus for the last nine years and, but I think that is an opportunity to really hone that discussion into strategic action.

Steve.

DR. GOODMAN: I didn't -- PCORI has been a leader in the open science, we're the first, I think, major funder to require the data sharing. But it would be interesting to see what the yield on that is. It's a theory that that results in more wider dissemination of the research products and use by other investigators. But we need to look at that. So we're very early into it, but it'd be nice to know how many data sets have already actually been posited. Has anyone asked for them or reuse them? Have there been any publications from those? I think it's too early right now to see much, but we should start actually formally tracking that.

CHAIRPERSON GOERTZ: Thank you. David, and then Michelle.

DR. MYERS: Thanks Dr. Briggs, I'm wondering if in a time of transition, one of the things you can help staff do as they contemplate 2.0 is rather than thinking about the big walls, the big, big challenges is take a moment to ask staff about the pebbles in their shoes that are stopping them from being effective and use this as an opportunity for a very safe place for what are the little things that we could do as a Board to help staff be more effective moving forward.

DR. BRIGGS: Thank you David.

DR. McMURRY-HEATH: Thank you. I also really enjoyed the presentation this morning. Just a quick follow on to Gail's point earlier, as we're thinking of kind of the white space of what could we be considering and studying going forward, I think we should also, it would be helpful to have a consolidation of the voices of interests. So what consolidated list of the stakeholder feedback, in terms of the PCORI's research and mandate would be

very helpful because we hear snippets like payers want more of this or you know, some patient groups want more of that. But having it in a consolidated compendium would be very, very helpful.

DR. BRIGGS: I agree. But also hard to achieve. We've worried a lot, very appropriately, about patient-centeredness, which is I think our first key stakeholder, but we have others. And how to bring what at times are not coherent interests of the other stakeholders in some ways a challenge for all of us.

DR. McMURRY-HEATH: That's a very good point. And you know, what comes to mind for me is what we've heard through the reauthorization process, to just having that distilled would be helpful, but maybe there are other pockets of interest that we might need to bring together to get more clarity. So at least they've been part of the synthesis process when PCORI 2.0 is unveiled.

CHAIRPERSON GOERTZ: Well, I think we have an opportunity to really reach out to our stakeholder groups during the -- during our

strategic planning process over the next year. I don't envision this as something that we huddled together over an over a weekend and, you know, come up with a strategic plan, but that we take a more thoughtful long-term approach and make sure that it is committed to having stakeholder input -- important and impactful stakeholder input along the way.

All right. Any others? Bob.

DR. ZWOLAK: The concept of refining the research that we support, I think is, is so incredibly crucial. We've had three big buckets. We've had the broad applications, the pragmatics, and the targeted and trying to determine within those three categories which were the homeruns, which may have been the singles -- I think it's going to be terribly important. Also, in fact, trying to define if those are the right three buckets to have will be helpful.

But the determining this, obviously it will be the ultimate work of the Board, but a bunch of it will fall to the Science Oversight Committee and the

associated staff and getting ideas from Board members, getting input from Board members, input from PCORI, all of PCORI staff, but in particular the Science staff and all the stakeholders is it's going to be an integrative process which is -- which will really shape 2.0 and I think Josie, you'll be able to help us begin to collect and understand all of that information.

DR. BRIGGS: Well, I will certainly try. I think you'll find the presentation, one of the presentations we have planned here today from Neeraj Arora, on our cancer portfolio, highly informative and one aspect of that portfolio is that it is not the response to talk primarily to targeted initiatives.

So it does tell us something about what actually comes together in what at NIH we would call more investigator initiated and that is part of the balance. It is when do we need to jumpstart something that isn't happening versus to what extent can pretty wide-open solicitations bring in particularly good work.

CHAIRPERSON GOERTZ: I agree Bob. I think that is an opportunity for us to evaluate because it is something that we've talked a lot about and now we should have enough data to at least get some preliminary information. So the understanding that some of our larger studies also take a little bit longer to execute on.

All right. Any other -- any thoughts or -- all right.

[No response.]

CHAIRPERSON GOERTZ: In that case we're going to move on to our next presentation, which is what we've learned from patient-centeredness and engagement. So we have three staff members that are joining us. Kristin Carman, Laura Forsythe, and Lia Hotchkiss.

* MS. HOTCHKISS: All right, thank you for joining us this morning. Now I'll let you get started.

Great. Thank you. All right, so for this presentation Kristin, Laura and I work together to summarize the PCORI's engagement efforts over the

past nine years. So in the next 25 minutes, we're going to take you on a journey from the PCORI's mandate from Congress all the way to current day looking into the future.

So we're going to describe for you how we've embedded engagement with patients and stakeholders into everything we do at PCORI and we're going to share with you what we've learned about engagement so far. You're going to hear how engagement contributes to and has an impact on research, individuals, and organizations, as well as the challenges and opportunities related to engagement. We would then like to spend time discussing with you all next steps for PCORI to build on those accomplishments for engagement, science, policy and practice.

So to begin, we're going to take you back to 2010, when PCORI was established by Congress to meet an unmet need. So they recognized that despite all the traditional research available, patients and those who care for them did not have the information they needed to help guide their decisions about the

healthcare choices they faced every day. So PCORI was created to ensure that research would be more accessible and relevant in order to ease the burden of healthcare decision-making. And we were intentionally labeled the Patient-Centered Outcomes Research Institute, and that has made a big difference. So we were authorized to conduct the kind of research that engages patients and stakeholders, and addresses questions that are important to them.

So the Board translated the mandate for patient-centeredness in some critical ways, including making it part of criteria for funding from PCORI and the research questions being studied and the outcomes included must be of importance to patients and those who care for them. And studies must engage patients and stakeholders as partners in the research process.

Disseminating and promoting the uptake of research findings is also a crucial part of PCORI's legislative mandate. PCORI is responsible for making research results useful, actionable, and

accessible for patients and stakeholders. So from the very beginning engagement has been fundamental to the structure and purpose of the PCORI and the Board and staff worked extensively to conceptualize how to achieve the goals set forth by Congress.

So the decisions about making patients, caregivers, and other stakeholders the North Star for PCORI were not aspirations but rather driven by legislation. So I'm now going to turn it over to Kristin.

* DR. CARMAN: Okay. I'm eternally apologizing for my voice. Christmas trees and other things have made allergies terrible, so I apologize for that.

So when PCORI was authorized there -- especially for those of you who have been here from the very beginning, you knew there was relatively little evidence about what engagement should consist of in research, the best ways to do it, and support it. And really the immediate impact on clinical research and the more distal impacts on health. So to execute on the complex mandate from Congress, you

heard about from Lia just now the Board and the staff developed a logic model or a conceptual model that would really help them understand how engagement would contribute to the ultimate aims of the PCORI. So this past model that you see here really built on ideas from other disciplines like community-based participatory research and shows how engagement helps to achieve research that matters to patients and those who care for them.

So the other elements of what's called the PCORI approach are shown there on the left, right? Which is the intensive portfolio management and obviously the investments in dissemination and implementation. The diagram is really how the Board conceptualized the problem and the model shows how we will know if it works, right? So if you have the PCORI approach, it leads to studies that matter to patients and that's in terms of quality and relevance and outcomes. And that includes topics and issues. And those outcomes by the way, are not just quality of life, but really where patients and other stakeholders and clinicians give input on what

are the key outcomes to us.

So from this model, then we go to the strategic goals. Things are useful. You speed uptake, you get more influence, which leads to the ultimate impact on health decisions, care, and outcomes. So from this model PCORI derived our evaluation about the PCORI; how to go about our work, including many important questions about how to support and effectively engage patients and stakeholders. I think what's really critical to understand as you hear this talk though, is that what might be the best approaches for ways of engaging patients and stakeholders really lack that strong evidentiary basis. So all these pathways seem awfully clear. The exact mechanisms were absolutely not. So that's why PCORI did not specify or dictate specific engagement activities, only that it must occur.

So this is intended to show you sort of the foundational aspect of engagement and this really shows that patients and stakeholders from across the entire healthcare enterprise, from payers to

patients and clinicians are involved in the entire research project all the way from sort of what topics do we fund? All the way through implementation and uptake. But I want to go a little bit deeper into this slide. There are truly diverse enriched stories behind every single number in this slide, but I really want to draw your attention is to the full scale of it, right?

So while as I mentioned, we did not invent the approaches or concepts of engagement. What PCORI has uniquely done is engagement on a scale and breadth and depth and organizational focus that no other funder has accomplished to-date. And just for example, there have been 410 awards to organizations and communities to build capacity both in terms of being involved but also to support uptake and dissemination, 850 unique organizations have participated in our workshops and convenings and 600 patients and other stakeholders have served as merit reviewers across our proposals for funding.

And while some funders might include a patient or a stakeholder in an opportunity, PCORI

has patients and stakeholders who participate as full participants on nearly every funding opportunity we have. This is genuinely unprecedented. And so when we use the term laboratory of learning, this is the laboratory that we are speaking about.

So we've tried to make the most of this natural laboratory by setting engagement in every aspect of what we do to develop a body of evidence. So starting from the bottom of the figure, the range of data comes from project reporting to externally led studies and evaluations, right? So that means we both conducted and commissioned observational studies about engagement in our projects.

And so, this is included over 120 confidential in-depth interviews with researchers, patients, and partners and that's patients, clinicians, payers and everybody. We've reviewed over 125 peer-reviewed articles. We've looked at hundreds of surveys, analysis of programmatic and administrative data. So it's a lot.

I want you to notice on the far right

though, the practice-based experience. PCORI also created the role of engagement officers who provide thought leadership and technical assistance to the projects and their work both generates research questions in real world as ongoing activities are undertaken, but also generates new evidence about evidence-based approaches, like a new six-month engagement milestone for projects to get feedback at an early project stage for engagement approaches.

So given the types of questions and data we have at this time, but we've primarily used advanced qualitative analysis. We do use mixed methods when we have the data in which to do it. And we've also created, obviously, peer-reviewed manuscripts and lots of project study reports.

The nice thing is we've been able to triangulate across many efforts and there really is a convergence on key takeaways which Laura is going to be sharing with you. I just want to note that our various analyses at all the different stages of the life cycle of her work really do surface concepts that resonate with each other. They both

add new information but they resonate with each other. They also echo concepts from similar, other kinds of projects and also conceptual models that tells us that we're building a robust database and that we really are triangulating with other concepts.

And I think it's also important to note is while it looks as though this learning has been very linear, as you all know, in reality this has not been linear at all. It's actually been quite iterative and an ongoing process. So with that I'm going to turn it to Laura to talk about what we've learned at least in a particular key area.

* DR. FORSYTHE: Yes, thank you. We have learned a great deal about engagement and so we have time today to share with you some of the highlights.

The first thing I want to talk about is how engagement makes a meaningful difference in the design and conduct of research projects. So we'll focus for the next few slides on that one key aspect and as we mentioned, we've identified several core themes across multiple efforts to study engagement

in PCORI projects. To give you a sense of scale though, in just our most recent study alone, nearly 400 distinct examples of the way engagement influences study design and conduct were analyzed from interviews with 60 different projects.

So when we talk about engagement influence on a project, what I mean is that it inspires or produces specific discrete decisions, behaviors, events, strategies within the study. What we've learned is that engagement influences study conceptualization, execution, and materials, as well as how study tasks are carried out. The way engagement is designed and practiced within projects and also researchers, the understanding of patients, clinicians, and the organizations that they're studying.

I'm going to focus more today on specifically how engagement influences study conceptualization and execution. We've learned that engagement influences all aspects of comparative effectiveness research projects from research focus to tailoring and delivering interventions, all the

way through dissemination. We're going to take a deep dive today on three aspects, specifically research focus, research design, and recruitment and retention.

You've probably heard a lot actually about how engagement influences research focus from determining topics and aims and comparators as well as outcomes or the constructs to study. I want to give you a few examples first, this is an example of a trial comparing medications and multiple approaches to exercise for back pain and this study included as primary outcome measures, physical activity and walking capacity. And the reason they did that was specifically because patients stressed the importance of maintaining their independence as their long-term preferred outcome. This particular project is an example of partners and researchers working together to develop their plans.

We also have examples though like this one where stakeholders have more of a redirecting influence. This example is the PROSPER study that compared different medications after stroke and what

the investigators shared is that they recognize through engagement that their original plans were too narrow. They ultimately added a primary outcome based on home time out of the hospital.

While the specific outcomes that are considered patient-centered really depend a lot on the population and the context being studied. What we've learned is that patients in general value outcomes about health status and wellbeing, about knowledge and understanding, and also about evaluation of their care.

I'm going to talk in a few minutes more about why these ways that engagement influences research focus are important. For now I do want to make a link between the notion of engagement influencing research focus and PCORI's ability to ultimately fund studies that matter to patients.

I guess it also influences research design. This includes things like very practical decisions about setting and timeline, as well as decisions about the number and type of study arms, and also defining broader inclusion and less restrictive

exclusion criteria.

One example is the TEAMS study, which compared different approaches to exercise for multiple sclerosis. And this study chose to randomize by clinics rather than by individuals to prevent treatment crossover. And this decision was inspired by stakeholder feedback about how patients participate together in support groups in their clinics. So these kinds of discrete decisions about design really have implications in multiple ways, including internal validity, external validity, and also things like the feasibility to conduct the studies.

Engagement also influences recruitment and retention. This includes things like designing the outreach strategies, as well as anticipating barriers and troubleshooting when unexpected barriers arise. Partners also participate in the actual activities of recruiting and retaining participants.

For example, the study you heard about in the Executive Director's Report comparing broad

versus narrow spectrum antibiotics for children. In that study, the partners help to create and design the consent script that they use to recruit parents as participants in the study.

Another example is this trial studying the effectiveness of self-management resources for children with diabetes. In this study, the stakeholder partners helped to ensure the study appealed to families who traditionally experienced disparities in management of diabetes. And what the team discovered was that as a result of this input, they felt like directly related to the input. Minority families were successfully enrolled in this study relative to other similar studies.

So I've shared some about what we've learned about how engagement influences discrete decisions, events and behaviors within study conduct. I'll move now to tell you a little bit more about how investigators and their partners interpret these influences in terms of why they are important downstream for the study. What are the downstream effects of these decisions?

These things that we've identified tend to be more practical and intermediate. These are the early steps on that path towards the ultimate impact of the PCORI's funded studies on healthcare delivery and outcomes. So again, we identified from multiple efforts and studies on engagement, several high level interdependency means about the way engagement shapes PCORI-funded research projects.

The first theme I want to share is about user orientation and acceptability. This reflects minimizing burden, maximizing usability, and really aligning studies with patient, caregiver, and clinician preferences, values, and needs. In short, this is about studies that patients and clinicians want to participate in.

Let me give you an example of that. One example is a trial comparing different approaches to facilitating providers and patients having conversations about the goals of their care when they are newly diagnosed with advanced cancer. So in this study, both patients and clinicians played a key role in ensuring the study was acceptable.

Among other things, one of the things that patient partners contributed to was reducing the survey response burden since patients with advanced cancer fatigue easily.

Oncologists played a key role in helping the study understand the practice landscape for this type of clinical issue and helped to refine the intervention. They helped to address oncologist concerns about the time involved and ensuring that the training and the intervention demonstrated respect for the patient-provider relationship. And this study noted that while it took them time in the design phase to do this engagement, that ultimately they had physician buy-in and stronger physician participation than their past experiences.

And you'll see the connection, I think, between this idea about acceptability and our next theme, which is feasibility. This is about mitigating real or potential roadblocks, about ensuring interventions, enrollment, and data collection are doable in real world settings and about achieving sufficient samples in terms of both

size and composition to ensure the conduct of the study.

So to just give you one example, I want to go back to the TEAMS study in multiple sclerosis that they talked about. The authors note that this is the largest trial of its kind conducted in multiple sclerosis and that they would likely be behind on their recruitment without PCORI is built-in upfront a commitment to engagement. They note that the study was set up so that there were built-in mechanisms to ensure they reached the finish line with a sample that would allow them to take this work to the next level.

Some of the other key themes that we learned about in terms of how engagement shapes studies include quality, which reflects the rigor of the study as well as the materials. Relevance, which reflects the results being applicable and important for decision-making and also the scope and quality of engagement.

I do want to note as well that not every discreet example of engagement influence on a study

is connected to a downstream effect. In some cases, not every decision was a big one and also in many cases it's just too soon yet for the people involved to know what the downstream impact or effect will be.

When you look across these themes though: acceptability, feasibility, quality, relevance, these are precisely the things that PCORI's legislative mandate tasked us with addressing in terms of the value of research for the people who are using it. These are also the elements that are in PCORI's conceptual model in terms of studies that matter to patients to put us on the path towards quicker uptake in clinical practice and an ultimate impact on healthcare outcomes.

So moving beyond the research itself, engagement also has value and benefits the people involved: patients, stakeholders, communities, researchers all find value in the engagement experience. Patients and stakeholders have reported that engagement increases their enthusiasm for research. It leads to new skills and professional

opportunities and particularly for patients, it helps them improve their personal health management and healthcare management and navigation.

Communities tell us that they build trust and build their research capacity. And researchers report that not only do they have a better understanding of the people and organizations they're studying, they also have a greater commitment to engagement in the future, whether it's required by their funders or not.

And moving beyond individual researchers. We also have evidence that PCORI's approach to engagement is helping makes a culture of research more patient-centered more broadly. And this is through catalyzing or inspiring change at other organizations and institutions. We have many themes of the ways that PCORI has influenced other organizations and we have multiple examples within every theme that we have detected.

This ranges from things in terms of building capacity, like seed funding and training opportunities, to incorporation of patient

reviewers, to enhancements in infrastructure, resources, policies and support for engagement in the design and conduct of research, all the way to other agencies and institutions adopting more patient-centered approaches. One example is the FDA's Center for Devices and Radiological Health developed and implemented a patient advisory council that was inspired by and modeled directly after the PCORI's advisory panel on patient engagement.

So I've shared a lot about what we've learned about how engagement influences research design and conduct, the people involved, and other institutions and organizations. We've also learned that engagement is not always easy to do. So Kristin's going to tell you more about what we've learned in terms of the challenges and opportunities for engagement.

* DR. CARMAN: I get the harder part of the conversation. You recall that as we talked about earlier, PCORI purposefully did not prescribe how engagement had to occur. Right? And so, it provided general guidance to the rubric and other

mechanisms. And I think there's no question even from what we found in our research that individuals with less experience and skill and comfort in engaging communities, engagement can feel especially challenging.

This slide really categorizes the types of challenges shared with us by PIs, patients, and other stakeholders. So let's start on the left and we'll move to the right.

So infrastructure and resources are pretty much just what you would expect to be problematic, but I think what's especially noteworthy is on the bottom is the time challenges. Time challenges everybody. It challenges the PI, but it also challenges the patients and the stakeholders who have lives, families, jobs, illnesses. And so, there's a real challenge about how to do this in a way, efficiently and effectively to get the voices you want to have heard and to really create that meaningful contribution that people want to have on projects.

In terms of the next column, people and

teams. Stakeholder-driven research truly is multi-stakeholder and with all the attendant challenges that come with multi-stakeholder research. I think this whole issue of integrating stakeholders with specific perspectives that's from the community and then the comparative effectiveness -- they all have their own specific perspectives and so there's always work to be done there. But I think what is really challenging everybody is really the diversity and inclusivity.

Learning about the live context of patients and stakeholders is probably one of the most important benefits of engagement for PIs and project teams. And everyone involved in the work is really concerned that the people participating, while welcome and necessary, do not always represent the diversity of individuals with the conditions or circumstances under study. And so, the consequences at times critical voices are not present at the table when they need to be and really from critical junctures in the projects.

So moving to organizations. You know, PIs

still have institutional barriers that are challenging for them. A good example are some IRBs who have not always understood the ways in which engaging patients and stakeholders as collaborators is really fundamentally different from them being research subjects. And PCORI, itself, has run into our own challenges to allow for the time and the flexibility to really maximize the input from engagement.

And moving to our right, this is really perhaps the greatest challenge. Laura mentioned that engagement is not always easy to do. It's not, it's also not always easy to live by. For example, RCTs, right? Which are seen as the gold standards and methods can be viewed as unjust or unfair by communities and they may be unwilling or concerned about participating. And it's not just patients who have differing views. You know, sometimes clinicians don't always agree that treatments really are or have equipoise.

So in reality, generating high quality, high impact research has many challenges, right?

Ethical reviews, safety assurances, getting to rigorous methods among others. And I think engagement, especially when new, is genuinely disruptive. But, and I think this is really important, participants in PCORI research describe engagement as an integral and valuable part of their projects, albeit sometimes quite hard, not wholly successful or unsuccessful by the way. But what it does do, it has helps to highlight looming problems and to solve many of the challenges of doing pragmatic real world research and the challenges that it helps to overcome really are precisely those that the legislative mandate sought to address as Laura mentioned.

So I want to take a moment to summarize. So what's been built from this time of this legislation with this complex mandate to where we are today? Well, from this authorizing piece of legislation, this is what the Board and PCORI has built. Opportunities for engagement in all aspects of the research process. General guidance on engagement, now developing more tailored guidance.

I mentioned that milestone document which PIs can use to refine their engagement plan six months into the project.

Of particular note are these two projects; one is research fundamentals online learning package and development of working as a team online tool to support teams working together on many of the issues and these challenges we've talked about are embodied in these toolkits to support these teams. We're also working on tools to facilitate clinician engagement, practice-based clinicians.

We also have a large portfolio of both capacity building and research projects that are at least sending signals that they're going to affect practice and ultimately health outcomes, as Josie sort of mentioned.

We also have a body of the science of engagement. It's burgeoning. It's not full, of course, but it’s impact on how to do it. We also have a change in culture in people and institutions who are interested in benefiting from engagement.

So what's next? Well, there are things we

can do right now to improve engagement and to enhance it. Starting on the left where the highest priorities is advancing our methods and approaches to ensure diversity. And one of the areas is in the topic generation, topic prioritization in our advisory groups, I know that came up earlier.

And then within projects, really supporting individuals to represent themselves and those like them as well as greater access for community individuals who aren't at the table right now. And I mean that in the broadest sense of communities and stakeholders.

We also need to leverage our evidentiary base to update our rubrics. And we also need to look at potential expanded opportunities with the Methods Council to think about should we be expanding standards and criteria for engagement in the future.

We also need to sort of make our engagement easier and more efficient, but we also need to think about what are the ways in which we can translate what we're learning into doing that.

We know we need efficiencies, we know people want to do this. And even when they don't want to do it, they still want to know how to do it as most efficiently and targeted as possible.

And this is really closely related to the next column, which is our science of engagement. We really do need more information about which engagement methods work best in which context. Obviously we need to understand better how to increase diversity. We also need to, and I think we have the data now, to think about how to measure engagement more effectively patient-centeredness as well as its impact. We also very much want to understand how engagement enhances dissemination and implementation.

We also need to start thinking about how do we map best practices and linkages of engagement into healthcare practice. Because we can do that now because we're going all the way from the studies now into linkage and uptake. So we have a real opportunity to think about that, although that's a little more of a future state given the state of

where our portfolio is at.

I guess the way I'd sum our progress on the study of engagement, it has both going great and is a little bit limited by our level and type of investment about how we study engagement. It would be really beneficial to develop an employ more innovative methods beyond the observational ones that we've had access to. We'd like to do more comparative, experimental and quasi-experimental study and studies and things like that, which would really allow us to get more robust answers to many questions. But perhaps the most critical answer for our communities who are trying to do this is what works for whom, in what context, and what resources are necessary in order to achieve it.

And finally, obviously we need to share what we're learning and innovate and how best to do it.

So we have a few discussion questions. These are merely suggestive, we rather expect you may have questions on your own things that you would like to talk about. So with that, I will turn this

back over to our Chair.

CHAIRPERSON GOERTZ: Great. Thank you so much for the really excellent presentation. We really appreciate it. I'm going to start with Ellen.

DR. SIGAL: Some really good work and I think we have much to be proud of. But my question comes to the uptake in the real world uptake of what we're doing and specifically do we have data on which advocacy groups that actually do clinical trials or incorporating this methodology? Industry? What are they doing about their trials? Cooperative groups? NIH, when they do their studies and investigator initiated research?

I don't see a lot of it in what in my world, so maybe I'm just not getting it, but I'm very involved in cooperative group clinical trials and clinical trials and data on clinical trials. And yes, there is some, a token patient involved in it, but basically these trials and industry's trying now to get patients from all. But I don't know if we could measure all these sectors that are really

doing the vast majority of the clinical trials and say that our research or our uptake has been substantial. And I'd love to have some data on that.

DR. CARMAN: We would to, and actually that question of how does PCORI compare in terms of its ultimate impact, speed to uptake, speed of getting the trials done. All of those have been a part of our evaluation framework since its inception in about 2013. And so, we share your interest in that and desire.

All along the way, we do our best to compare PCORI's work to publicly available benchmarks, although they are hard to come by. PCORI is a trendsetter in terms of transparency and evaluation and sharing openly how we think we're doing. So we continue to do that.

We also as you may remember, are working hard on developing a dataset with publicly available data to compare PCORI to public and private funders, industry, NIH and other funders, as well in terms of things like efficiency of recruitment, time to

publication, things along those lines. So we are working on that now and we will share that with you as soon as we can.

One thing to keep in mind is for those questions to be answerable, we need a little more time for PCORI's studies and our peers, to which we are comparing, to get to the point that we have a body that is to that maturity in terms of uptake. But we think there are some indicators along the way too in terms of things like how long it takes to get published that we're at the ready to track.

DR. SIGAL: There is data --

DR. McNEIL: Oh, I'm sorry.

DR. SIGAL: I'm sorry.

CHAIRPERSON GOERTZ: Oh, okay Barb. We're going to go around the table and then we'll get to you. Okay.

DR. McNEIL: Okay, sure.

CHAIRPERSON GOERTZ: Thanks.

DR. SIGAL: I just think there is a fair amount of data available now from NIH and from industry. And again, everyone in some ways is

incorporating patient input, but I don't think it comes close to the standards we've set. So I think there is a fair amount of data that we can capture now and I'm skeptical that, you know, 10 percent of what we're trying to do is being done. And particularly, when you get to the academic centers that are doing investigator initiated trials. And many of them, many of us would never go on.

DR. CARMAN: Yeah. We're working on capturing all that now and look forward to sharing it with you.

DR. FRIESE: So I've been involved for over 20 years with Project LEAP, which you probably know about. It's the National Breast Cancer Coalition, which conducts between two and this is for everybody else who may not know -- two, five, and even longer day workshops and institutes to train in this case, breast cancer patients to be advocates, partners, DSMB members, protocol reviewers, et cetera. And it's very intensive and they bring in scientists from all around the country. And this dramatically increases the quality of their involvement because

they can speak the language and meld their own perspective with the language and perspectives of the scientists.

Do we do anything of the kind? And if not, could we?

DR. FORSYTHE: So I'm really happy to say that through the engagement award program, we have supportive project lead to incorporate some of the methods that we've been talking about today into that training that they do for breast cancer advocates. So specifically we have been working with NBCC.

But the engagement award program at PCORI really has been fundamental in providing support for the development and implementation of these sorts of training programs, not just with NBCC, but many other patient advocacy organizations who want to create their own communities of stakeholders who are trained in, interested in, and able to partner in research.

So I'm also happy to share that in September we launched Resource Repository on the

PCORI website and includes a lot of these trainings and tools that have been created through the engagement award programs that organizations are using to build up this capacity. And so there's a lot of more publicly available resources that others can use to do these sorts of trainings.

DR. FRIESE: Can I just push you a little bit on that or understand better.

DR. FORSYTHE: Yeah.

DR. FRIESE: Do these involve in-person training over multiple days or are they online resources?

DR. FORSYTHE: So we fund through the engagement awards, the project itself is a two year project up to $250,000 to actually do the in-person trials.

DR. FRIESE: I see.

DR. FORSYTHE: Yeah. And because they're also different, we've made over 450 awards now. There's no one gold standard, for what a project looks like.

DR. FRIESE: Right.

DR. FORSYTHE: So we've seen many variations. Some do it more in-person, some do it more online, but there's a whole spectrum of the types of resources that can be used and have been used.

MS. HOTCHKISS: And just to be, to make sure we're being clear to connect it back to the projects. Remember at the project level they weren't required to do such a thing. Some might, in fact -- some have created their own toolkits. Part of what we've been doing with now -- as I mentioned, the two online learning packages and particularly the team package is really trying to -- from what we've learned about what works in our project and frankly what doesn't work, is create various approaches.

So that might suggest you do it online. It might suggest go off and do this as a two-day team training.

CHAIRPERSON GOERTZ: Thanks. Gail.

MS. HUNT: The thing that has struck me is when we talk about -- when we actually get down to

using engagement to disseminate. And there was, I guess it was slide 41 is the one that I was interested in.

We don't talk about, you know, we've involved the community and we've involved organizations say patient advocacy organizations, but we don't talk about going back to them to help to disseminate the information. So instead of it just going through JAMA and those areas, it should be going back, whatever it is, going back to those organizations that really care about this and are willing to push it out to the patients who are their members.

DR. FORSYTHE: And Lia can talk about it. I just want to note that what we've talked about here is that we actually do a lot of that. We, it's more recent so there's less evidentiary base about it's impact, but I think Lia can tell you more about sort of the dissemination piece. But we also, we do go back to those communities.

MS. HOTCHKISS: Yeah, that's right. And about a year and a half ago, we put out a funding

announcement that the type of engagement award specifically focused on involving communities in being our dissemination arms for our research results. It was called our Dissemination Initiative and it was wildly popular and we have over 20 projects underway right now where they're community-driven projects to actively disseminate our research results.

So we'll have some more data to share soon, but these have really just been very popular and I hope that we'll be able to continue to fund those types of activities.

DR. FORSYTHE: And within the dissemination and implementation projects, it's a critical component to demonstrate who needs to be participating in that project in order for this project to either be disseminated or implemented. So I think this is being carried through. We'd very much hope to have information for you, more evidentiary information of the impacts of including these communities. We just need a little bit more time given where we are in the life cycle.

CHAIRPERSON GOERTZ: Thanks. Barbara.

DR. McNEIL: Yeah, I thought the presentation was very interesting. I had one question about the second presentation, which involves the role of patient input into various parts of the experimental process. And the question I had is, do we have a sense of what the percent difference is of the effective patient involvement in the design of the research project or in the extent to which the outcomes differ as a result of their input? What's the relative balance between the inputs for these two -- for these two parts of the process?

DR. CARMAN: Yeah. Barbara, can you repeat it? It's a little bit fuzzy over the phone and I want to make sure I'm answering the question that you're asking.

DR. McNEIL: I'm sorry. Hello? Can you hear me now? Better?

DR. CARMAN: Yep.

DR. McNEIL: Okay. So what I was asking was it was several parts of the process that you

mentioned in which patient involvement could play a role. The second one was in the design of the experiment and the third one was in the description of the outcomes that were measured, as I understand what you said, is that correct?

DR. CARMAN: Yes. Yes.

DR. McNEIL: Okay. So what I was looking for was that the extent -- what was the relative percentage of importance between the second and the third? How often do they place a valuable role in the design of -- and the one about MS was pretty compelling, but we might have thought of that ourselves if we had a good designer.

And the second one is how often did they actually change in a really significant way, not a trivial way, the outcomes that are measured?

DR. CARMAN: There's a couple pieces that I want to make sure we hit on. One piece is we didn't spend time on this, but one important thing we've learned is about the different paths or ways that engagement has an influence on our project. And in some cases you might conceive of it as a change or a

redirection.

But in many of the cases, this really is about people coming together at the beginning of an effort to say, what do we need to study? And so, because they are designing it together we don't, we can't answer that question about what would it have been. And it is unknown to us as an organization and to them, because they were together from the start.

We do have examples of both of those paths, but increasingly over time by design, our awardees are shifting much more towards that co-production or design as opposed to researchers having an idea of bringing it to somebody and then they say, well, can we get like this or like that.

So I will say that the focus on outcomes as really defining what are we going to study here is one of the most common themes that arise across our efforts. It's a key area.

DR. CARMAN: I would also say I think your intuition is right. I think Barbara, where you're going is that I think it's, I think it's safe to say

that you're more likely to get larger changes in outcomes for a variety of reasons which we could discuss the major design changes, right?

DR. McNEIL: Right.

DR. CARMAN: That is both. I mean, I think that is fundamentally true, but it is also true. What Laura says is that for some projects it's the integration of the stakeholders and the PIs is so vague that they can't tell you because they sort of co-designed it. That doesn't happen with everything. But I think you are right that there is more emphasis on the outcomes for a variety of reasons.

Although there are what you might call more tweaks to designs, much more around the edges and then really radical changes.

DR. McNEIL: Okay, that’s what I was sold but I just wanted that clarified. Thank you.

DR. FORSYTHE: Yes. And one other thing I'll add is that when we think about design, you can think of it as a really broad umbrella term, too. So there were other things that were more common,

maybe not a major change to whether it's going to be this kind of trial versus that kind of trial. But some important decisions about things like the inclusion and exclusion criteria that, in particular, partners felt like were really important and the extent to which they were going to really be interested in the results and how much they reflected the communities they were intended to help.

DR. McNEIL: Thank you. That was a terrific response.

CHAIRPERSON GOERTZ: Thank you. Sharon.

DR. LEVINE: This is terrific work. Congratulations to the entire team. It's very impressive to sit back and listen now to the whole.

How much of this have you published? It seems to me there's a rich amount of material here when we talk about transforming the research enterprise, being able to demonstrate this.

DR. FORSYTHE: We've published a lot of it and I'm not sure we mentioned there is -- the last slide here is a select set of key publicly available

resources that anyone listening can go read about the details of the things we covered and some things that we didn't cover. Like what we've learned about engagement in merit review and some of the other aspects we didn't have time to get to.

We're also still actively working on getting more of it out there. Some of -- we are talking today about really a synthesis across a number of projects over the last seven plus years and there are some that are that are hot off the presses that people heard about at the annual meeting and other settings and there's some extra detail as well that we want to get out there.

DR. CARMAN: [Off microphone] ideas and thoughts about this. Because that's part of what we need to do is not just the individual publications, but a broader statement that’s sort of what we think we've learned.

DR. LEVINE: And what about convenings of scientists.

MS. HOTCHKISS: What a wonderful idea. Yes. We have had that conversation and something

we're looking and thinking about for next fall. But obviously, you know, there's things that --

DR. LEVINE: No, I don't mean your convening scientists. I mean, insinuating this work and yourselves into --

MS. HOTCHKISS: Yes going out, yes. We, in fact, we have an internal -- forming -- subcommittee on what we're calling translational work to sort of say, how are we going to get this out into the world and what are going to be our mechanisms across our teams. Exactly, the right question. Thank you for clarifying.

CHAIRPERSON GOERTZ: Thanks. Larry.

MR. BECKER: Thank you very much. That was terrific. So Lia, I'm going to, yes. So uptake and implementation, probably the hardest thing is to get these things actually in place actually being done.

And so I wanted to put in a plug for developing a comprehensive toolkit to put the horsepower behind it, the money, the time behind it. So that it focuses on different stakeholder groups, different environments, different settings,

different policy groups.

I mean, for example, how do you get changes prioritized for adoption in a hospital, in a doctor's office, at an insurer? How do you get those things done? And can we help the people on the ground, the patients, their providers, everybody to not have to reinvent the wheel every time. Maybe they have to customize the wheel, but they don't have to reinvent it and they can start somewhere because it's really hard, you know, to get a health system to change. They've got a thousand other priorities. Why is this one important and how do we get there?

So I just want to put a plug in for making our mark in beginning to solve the problem of we got all this great research. We've spent literally billions. Now let's make it work.

MS. HOTCHKISS: Thank you for that comment and I think that we're putting in place the infrastructure to be able to capture what's working for dissemination implementation, and what settings, with what stakeholders, how -- what was the finding.

And so, Joanna Siegel and I've been working very closely to start to track all of that so that we are collecting some data so we can down the road be able to compare and put together some resources. But that's yeah, that will be another synthesis, right. But not between the engagement work but between the planning for dissemination, the actual dissemination work that I think we'll be able to do in the future.

DR. CARMAN: But I will make another connection to that, which is what I hear you saying as well, Larry, which is that thinking about which we had talked about, sort of how we make that linkage from what we're learning in engagement and research and how to conduct these studies and uptake is also an issue of how consumers and stakeholders interact in the healthcare delivery environment. So who's making decisions about which things get prioritized and how they get prioritized.

And one of the areas that we spend a lot of time thinking about is that sort of what are we learning and how does that and should that influence

how we think about engagement and the care side. Because that's really at the uptake side is sort of what their role and relationship, not just dissemination but what's their role in the process of sort of deciding what needs to be prioritized. So we see that as something as for the future state. It's really important to be looking at as well.

CHAIRPERSON GOERTZ: Thanks. Robin.

DR. NEWHOUSE: Yes, just a reflection in two areas that I didn't hear. You mentioned that I think you've had an impact. And just to reflect back to where we began and how many times at the public board meetings that the public policymakers, the organizations were asking for engagement and the work that the Methodology Committee did toward the first set of methodology standards. It was very clear the patient-centeredness needed to be one of them.

There were strategies to engage the public multiple times in the formation of those standards that, of course, are now accepted. So I'm just saying that we not only are promoting the science of

engagement, we practiced it our self. And to see where you are now is just amazing. So it feels like if we were doing a summative evaluation, we'd say if this was the, you know, the 10-year plan, we're completely on the right path.

And then in terms of other kinds of impact, I was thinking about all the Rs that are funded through agency for healthcare research and quality. There's R24s that were funded, which were research building. There are K12s, they're learning healthcare systems. Each one of those Ks or Rs have a requirement to include these standards, which includes the patient-centeredness standards. So that is a dissemination arm for the patient-centeredness standard and I don't want to lose sight of that because they've had some great results and great engagement of new students of all types.

And then the last one is really just about being a faculty member and a faculty member who teaches research. And in the academic arena, there's a lot of discussion about scholarship for public engagement and what it looks like. And I

have to say, when you all publish an article, I make sure I get it right away because it helps to discriminate the difference between a story and methods and research and a program of research that focuses on patient engagement.

So I think that it is incredibly important as we train the next generation of our students to help them think about this because it is a science and they can have a very good career in understanding the methods of patient engagement and you're just a stellar example. Your papers are excellent.

CHAIRPERSON GOERTZ: Thanks. Mike.

MR. HERNDON: A little bit of a different comment, but I think it needs to be shared.

Do we have some awards for a local entity that has Mary's or Mary in the name? It's a research study that impact several areas where people can go for healthcare that are uninsured or under-insured. Does anyone know? Does that ring a bell in PCORI?

Yeah, it has Mary or Saint Mary or

something in the name. But anyway, I just thought this was worth saying because yesterday -- because of the impact. And by the way, great presentation, great comments. I appreciated that very much.

On the way back to the hotel from lunch yesterday, a very engaged female driver was driving my wife and I, and she just kept pressing on why I was in town. And eventually I had to give her the name, PCORI and I said P-C-O-R-I, and she said, “Oh, I’ve been to a clinic where they're doing work with that group.” And she said, “I've never had anyone care for me like these people cared for me.” So I may have the name wrong --

UNIDENTIFIED SPEAKER: There is FQHC work here in DC.

MR. HERNDON: Yeah. But anyway, and my wife just reached over and kind of patted me on the leg like, wow. Wow. There’s a real life example of how -- of what we do. We talk in theory and we talk research, but it's kind of cool every now and then to have anecdotal story that says it's working.

CHAIRPERSON GOERTZ: That's a great story.

Thanks for sharing. Any other final comments or questions?

[No response.]

CHAIRPERSON GOERTZ: Well, I want to thank all three of you once again for just an excellent presentation and a great, great discussion. We actually have -- we're a little bit ahead of schedule, but we're going to use that as an opportunity to extend our break. So we are going to resume again at 11:30. And so, those of you on the phone, that's what we'll be starting again. Thanks.

[Recess.]

CHAIRPERSON GOERTZ: All right. Before I start want to acknowledge that Janet Woodcock and Michelle McMurry-Heath have joined the meeting. So welcome.

So I'll now turn it over to Neeraj Arora and I'm Sarah Daugherty for a presentation on PCORI's cancer research portfolio.

* DR. ARORA: Thank you Christine. Good morning. Many of my colleagues have asked me to break a leg, but I have no intentions of doing that.

[Laughter.]

DR. ARORA: But I would say that along with Sarah we are extremely delighted to present to you and give you an update on our cancer portfolio. We hope that this is a first of multiple interactions that we will have with you over time to not only understand the impact of the PCORI 's investment on patient-centered cancer research, but equally important -- importantly to get your guidance and input on next steps and be forward on PCORI's investments in cancer.

So as a quick outline, we will begin with a brief background to, you know, put our work in a larger context and then we'll move on to providing you with an overview of the PCORI's cancer CER portfolio. We then will take a deeper dive and highlight some of the findings of some of our early studies that have been completed and also highlight some large ongoing pragmatic studies that are happening currently in the country addressing a variety of where decision, dilemmas that have been identified by stakeholders during different phases

of the patient's cancer journey.

And finally, we'll end with a few summary observations on our portfolio and hopefully engage with you in getting your guidance on future direction.

DR. SIGAL: I don't mean to interrupt, but I'm interrupting. I have a conference call on a Senate briefing tomorrow at 12. When will you be finished? Obviously this is an area of great interest to me. What is your timing?

DR. ARORA: So the presentation's about 25 minutes and then we'll have discussion.

DR. SIGAL: I won't be able to be part of it, I have an half an hour conference call that I have to be on for a Senate briefing, but we can catch up later maybe.

CHAIRPERSON GOERTZ: Okay. Absolutely. We'll make sure that we're able to catch up afterwards.

DR. SIGAL: Yeah. Obviously I have some thoughts on your thoughts on this.

DR. BRIGGS: Ellen your thoughts on this

will be very valuable. I think much of this you've seen already because it was part of the preparation for what we did last week.

DR. SIGAL: If you make them -- let me know.

DR. ARORA: Obviously we'd love to get your guidance on this.

Okay. As a quick background, as we know that cancer is a family of diseases with a high prevalence in the United States and currently it is estimated that 1.6 million people in the country are diagnosed with cancer every year. The figure, as we can imagine is with the aging of the population is likely to increase significantly.

At the same time, we also know that individuals diagnosed with cancer today are living longer than ever before and by 2026 it has been projected that there will be over 20 million individuals living with a cancer diagnosis in this country and more than two-thirds of those would be individuals 65 years or older.

Yet at the same time, we know that cancer

treatment trials typically exclude the elderly patients as well as those with comorbid health conditions. In fact, in 2013, the Institute of Medicine and now the National Academies of Medicine, of course, came out with a landmark report on evaluating the quality of cancer care in the country. And they explicitly stated in that report that future comparative effectiveness research studies should focus on patient samples that mirror the age distribution as well as health profiles of those who are living with cancer. So they were calling for more real world trials. The type of which PCORI obviously funds.

We also know that family caregivers often face a significant burden caring for cancer patients. It is important for us to not only focus on outcomes that matter to patients, but when possible also include family caregiver outcomes in our studies. The IOM report in 2023 also identified as a goal for high quality cancer care delivery for the future to be one that is first and foremost patient-centered, evidence-based, and well-

coordinated. Obviously that resonates very well with the PCORI's mission.

So now let us tell you about, I'll give you an overview of what we funded in a patient-centered cancer research.

So to-date, we have made it a funded 80 CER studies that amounts to an investment of over $300 million, which is roughly about 17 percent of the PCORI's overall investment in CER, both in terms of dollars as well as number of studies. And I would also note that while you know we are focusing this presentation on our CER awards, we've also made additional investments in the cancer space by way of several engagement awards, dissemination, and implementation studies, one PCORnet study as well as we recently updated two systematic reviews.

On this slide, provides a distribution of the PCORI studies by the different funding announcements. As you can see in this pie chart with the blue area of the pie more than three-fourths of our studies have been funded in response to our broad funding announcement where

investigators have worked with stakeholders to send us proposals on a variety of important decision dilemma that we have funded over the years since 2012.

Our pragmatic clinical studies program started in 2014 and since then we have funded 11 large pragmatic trials in cancer. This amounts to 25 percent of our entire PCS investments. And finally, in the green pie that you see on the chart, shows that seven studies have been funded in cancer that are part targeted funding announcements.

I would note that these are part of our funding announcement in palliative care and symptom management that were not focused on cancer alone.

So to-date because PCORI has not had any targeted funding announcement that focuses exclusively on issues of cancer. Yet at the same time, we have been fortunate to have a large portfolio of studies in our cancer portfolio. Next slide.

This slide is shows the distribution of our CER studies by the PCORI's four priority research

areas. As you can see here, almost half of our studies are clinical in nature, focusing on the assessment of prevention, diagnosis, and treatment options priority. A fourth of our studies are focusing on healthcare and delivery-related interventions.

And I want to note here that almost one in five of our studies are focused on our communication and dissemination research priority area. This is a little larger proportion of the portfolio compared to some of the other portfolio presentations that you've seen before. And that's largely because we funded many studies on communication and shared decision-making in cancer treatment.

Studies in our research cancer portfolio focused on a variety of cancer types. Majority of studies are focusing on the more prevalent cancers such as breast, lung, prostate, and colorectal cancer. And you can also see that the bar at the bottom shows that we have a large number of studies focusing enrolling patients with multiple cancers in their patient samples. These tend to be typically

our studies focused on health systems and communication interventions because often those interventions are invariant to the particular specific cancer type.

Some of the key portfolio characteristics that you would like to highlight is, you know, of the various studies we've funded, 70 percent of randomized controlled trials. Of these, a large majority of them are enrolling patients from multiple sites. Many of them are being conducted across multiple states. More than half of our studies include large patient samples, so more than 500 patients are being enrolled in those studies. And 25 percent of all our cancer studies are also enrolling caregivers and focusing on caregiver outcomes in addition to patient outcomes.

As we heard in a previous presentation from my colleagues on stakeholder engagement our cancer awards have also benefited significantly from engagement from patients and other stakeholders. Our PIs, based on the surveys that were done by our engagement colleagues, more than 80 percent of them

say that stakeholders have played an important role in planning of the study and finalizing the interventions, as well as helped significantly with recruitment and retention of study participants.

Similar to what we see across our portfolio, over 50 percent of studies include a patient and stakeholder co-investigator in addition to having them involved as part of stakeholder advisory committees.

Studies in our cancer portfolio are also focusing on a range of clinical and care delivery and communication interventions. For example, many studies are conducting head-to-head trials of different treatment modalities. Several of our studies are evaluating decision support tools, both for the patients and clinicians. And then, we've also funded many studies looking at different models of care delivery, both in the treatment and post treatment survivorship phase of care.

While synthesizing our portfolio, we decided to take a patient-centered approach to our analysis. We wanted to look at where in the

patient's journey are our studies intervening in order to optimize patient outcomes. So for sake of convenience, we classified the patient's journey into three categories for this presentation. As you can see here, we funded about 18 studies in the prevention and early detection phase of care. We have 50 studies in treatment with about 15 of them focusing exclusively on issues of care delivery for patients with advanced cancers. And finally, we have a few studies in the post-treatment survivorship phase of care as well.

This slide just shows examples of some of the topics that are being studied by our studies across the three different phases of the patient journey. The ones that are in bold are the ones that we will look at in detail when we talk about findings from existing studies.

So examples, for instance being, as you can see in the prevention phase, a lot of our studies are looking at how we can increase uptake of evidence-based interventions. For example, HPV vaccinations, colorectal cancer screening,

especially in populations that are at high risk for health disparities.

Other studies are focusing on facilitating shared decision-making between clinicians and patients while evaluating different treatment options. And then, in post-treatment survivorship, several of our studies are looking at the optimal models of follow-up care so that we can facilitate early detection of a recurrence on new primaries as well as late effects such as cardiotoxicity.

In terms of results, to-date out of the 80 studies that we have funded, 45 of them have completed their research and many of those have completed either the PCORI's peer-review process or have published their results and some of them are currently in the peer-review phase.

Now I'm going to hand it over to Sarah to take a deeper dive and share some of the results from our early --

DR. McNEIL: Chris. Is it possible to ask questions at this point or should we wait till the end?

CHAIRPERSON GOERTZ: That's up to Neeraj, would you --

DR. ARORA: Sure. Go ahead.

CHAIRPERSON GOERTZ: Go ahead, Barbara.

DR. McNEIL: Well, I really -- I think it's an important question, particularly related to what you're talking about and in the future. You talked about the fact that 15 studies involved studies of primary care, radiation surgery or whatever, and eight involved screening. You also mentioned on a prior slide that 75 percent of all your studies were randomized.

So my question is of the 15 and the eight, what percentage of those were randomized? That's really what's key in cancer. If we take apart -- if we put aside anything involved with how the delivery is provided. So basically what percent of 15 and eight are randomized? So maybe you could look that up while the next presentation goes on.

DR. ARORA: So Barbara I didn't get the question. Are you asking the question -- studies in the post-treatment phase?

DR. McNEIL: Well, if you go back a couple of slides, if you go back a couple of slides -- early on you said 75 percent of your studies involved were randomized and then this slide --

DR. ARORA: Oh, so we saying -- yeah, the 70 percent are randomized. So the 15 you were talking about, are those in the treatment phase that we have there?

DR. McNEIL: Were they randomized? No, I'm wondering about the 15 -- that was in treatment, I'm sorry, 15 in treatment and eight in screening. And what percent of those were randomized? That's the key question.

DR. ARORA: So yeah, several of our prevention studies are randomized.

DR. McNEIL: Prevention. Hold on. Wait, hold on. Hold on. Hold on. Where's prevention? This says treatment and screening? Where’s prevention? I'm sorry, I'm not there. Well, I can't, I'm looking -- I’m on the line so I can't see what slide. If you go back a couple. Can I go back a couple? This one says 70 percent are randomized.

DR. ARORA: Right.

DR. McNEIL: Right. Now go forward a few slides. Next slide. Next slide. There's another side. Next slide.

DR. ARORA: We haven't been -- gone to that.

DR. DAUGHERTY: This is as far as we've gone.

DR. McNEIL: Oh, then maybe go back.

[Simultaneous discussion.]

DR. DAUGHERTY: -- related studies Barbara?

DR. McNEIL: No, what I want to know is what percent of all of the studies that involve treatment or screening are randomized. Are randomized.

DR. ARORA: We can definitely, I don't think I have the numbers right now with us, but we can certainly go back and take a look at that. We did --

DR. McNEIL: I'm emphasizing that because I think you've done some great work, but I'm talking as a physician now, not as a researcher. If I talk

about what's important, the most important thing for my patients with cancer is the ability to know whether Treatment A is better than Treatment B. It’s also important to know whether the way it is delivered is critical and it's important to know a bunch of other things, but I really want to know is A better than B in terms of survival, symptoms, and side effects.

And you get those data by randomized study. Just think about how all a new PD-1 and PD-L1 studies are being done. They're all randomized. So that's all I want to know.

DR. ARORA: Absolutely. And Barbara, as you will hear when Sarah presents the findings from our early studies, we did fund several observational studies in our initial rounds of funding and those are studies that are completed. And we will highlight one, sorry, that's a trial. And the other studies are indeed observational studies. But then we are also going to highlight some of our ongoing large trials and almost all of those are randomized, so you know but we can -- we'll definitely take a

deeper breakdown of our studies by study design by each of the phases of care.

DR. McNEIL: I think that would be great. Christine, I think when we're talking at future meetings, we seldom indicate in the slides the trial design and that's pretty critical. So I would recommend going forward for all studies that are presented we indicate the study design in one little bullet.

CHAIRPERSON GOERTZ: Okay. Thanks. Thanks Barbara.

All right, let's go ahead and continue with the presentation.

* DR. DAUGHERTY: We'll continue here with our study spotlights across the patient journey. This is an opportunity for us to give you a fuller picture of some of the studies that we've funded. As Neeraj mentioned, the four studies that I'm going to be talking about are completed studies, three of the four are observational studies, and all of them have published their primary results in journals.

As Neeraj mentioned, our pragmatic clinical

studies program got started in 2014, so we will also be highlighting several ongoing clinical trials. These are larger, longer in duration, and more complex. And we will be summarizing some lessons learned from those studies that are in progress.

So the first study that we'll be talking about today is within our prevention and early detection category. This study is focused on increasing colorectal cancer screening among Hispanic primary care patients. We know the colorectal cancer screening is associated with significant reductions in colorectal cancer mortality. And while the uptake of colorectal cancer screening in the U.S. is about 63 percent, among Hispanics, it's considerably lower about 35 percent in this particular region where the study was conducted.

So Ronald Myers and his research team, were interested in asking the question of whether active decision support with patient navigator could improve colorectal cancer screening rates among Hispanic patients compared to mailed materials.

This study team was building off of a body of literature that suggests that tailoring of decision and navigation support by incorporating patient preferences and addressing personal barriers can actually help affect behavioral change. So this is a randomized controlled trial at five primary care practices in the Lehigh Valley Health Network in Eastern Pennsylvania. Their primary outcome was screening adherence at 12 months.

So what they found was that telephonic decision and navigation support increased colorectal screening compared to mailed information and they saw this both with respect to stool blood tests as well as colonoscopy. So these findings may suggest that this type of decision support can really help address screening disparities.

We pulled a quote from Dr. Meyer's project monitoring report. He, here in his quote, suggests that engagement really does help to enhance the intervention and more specifically he comments on how engagement has helped with the cultural appropriateness of the language and also engagement

helped with initiating additional support services, particularly financial counseling. Then ensured that the implementation of the intervention went well.

The next two studies that I will highlight here are in our treatment category. Both of these studies are comparing different clinical options for what we might consider to be preference sensitive clinical decisions. And that's in part because the clinical options have important trade-offs. The first study will be with respect to prostate cancer and the second with respect to breast.

So this first study by Penson and his research team is looking at what are the side effects of treatments at three years for localized prostate cancer. This is an important study because it continues to characterize the trade-offs between immediate surgery or immediate radiation and active surveillance or monitoring.

Now, there was an important trial, a European trial that was published in 2016 comparing immediate surgery to active surveillance and it

provided an important clinical outcome. However, the trial was started in 1999 and so the treatments that were evaluated are considered to be outdated here in the United States and the population was relatively homogenous.

So this study compares modern prostate cancer treatments that are standard here in the U.S., today, with active surveillance in a diverse population. They had 25 percent of individuals who were self-described as nonwhite. This was a prospective observational study. They identified participants from five SEER registries and the Capture Registry, which is supported by a network of community urological clinics.

The primary outcomes of interest were functional outcomes, sexual, urinary, and bowel outcomes as well as quality of life. And what they found was that men who had surgery reported a greater decline in sexual function and worse urinary incontinence compared to men who had radiation or active surveillance at three years.

They also found that men who had radiation

reported worse bowel function at six months. However, they didn't see any significant differences at three years. They also, as I mentioned, looked at quality of life, no significant differences and self-reported quality of life at three years. And they also looked by race and they found no significant differences by race except for one functional outcome, urinary incontinence where African Americans reported a greater decline compared to Caucasian men.

So this suggests that there are important functional outcomes as a result of immediate surgery that need to be considered in the context of some of the other information we have in the literature.

DR. SIGAL: I just, I again apologize that the timing of my call is horrible. There's a lot of work that has to be done in this and this is just the beginning.

First of all, the biology of the disease is very different and this is probably one of the most over-treated disease we have and the options are really complicated. So, you know, not now but we

should talk about this, but we have to be very mindful that this disease is very different in different people and some people absolutely need a breadth of treatments. How we determine this and how we do this is really important. Particularly now that we know genetic factors and we know how to screen. And so, there's a lot of opportunity for us to revisit this in ways that can be very useful.

DR. DAUGHERTY: Good. Thank you for that comment.

DR. McNEIL: So can I ask a question if you've finished responding to Ellen?

CHAIRPERSON GOERTZ: Yes. Barbara, is it just a brief question cause otherwise I think we're getting close to the end of the presentation.

DR. McNEIL: Well, I just wanted to ask and maybe it goes is part of a general discussion. I wonder how often we found the generic quality of life questionnaire useful for studies like this one, when there were quite major differences in patient reported outcomes.

DR. DAUGHERTY: So the question is about

how useful it is to evaluate generic quality of life.

DR. McNEIL: Correct.

DR. DAUGHERTY: I personally think that it adds to the understanding of the life experience. I think they were capturing important functional outcomes and an overall assessment of the quality of life added to the fuller picture of the patient experience.

I think it was also, you know, in the trial that was conducted, it's a common measure as well. So I think you've seen this in multiple studies for prostate cancer.

Okay. So let's move on to the next slide then. This is -- I thought you would be interested in some of the dissemination activities that have taken place, that have extended the reach of these results.

The first is that the PCORI has funded a dissemination implementation award to update an existing prostate cancer treatment decision aid with the more representative data generated by this

PCORI-funded study. This is an important next step because it provides population-based estimates that now can be updated in the algorithms of this particular decision aid.

The second activity is demonstrating how we have partnered with clinical and consumer groups to raise awareness. So the second bullet here is partnering with -- showing how we've partnered with the Men's Health Network to co-host a Congressional briefing on shared decision-making for prostate cancer. And the third bullet here highlights the development of online continuing medical education activities that highlighted both the Penson study that we just talked about and another PCORI-funded study by Ronald Chen that describes the contemporary treatments for localized prostate cancer.

Okay, so this next, this third study, we have four completed studies. So we actually do have a few more to talk with you about if you have the time.

This third study is focused on an important decisional dilemma that women who are diagnosed with

breast cancer in one breast face with regard to various surgical treatment options. Their treatment options include removing both breasts, the cancer breast and the healthy breast versus removing part or all of the breast with cancer, but keeping the healthy breast.

Now for women, I think as Ellen was suggesting there are some women who are considered to be at high risk for developing a second breast cancer in the healthy breast. These women have a strong family history. They often have genetic mutations and they commonly will choose to remove both breasts. Women who have non-familial breast cancer or what would be considered sporadic breast cancer, have much lower risk for developing a second breast cancer in the healthy breast. However, we're also seeing an increase in women who choose to have both breasts removed in that population.

So while there may be a number of factors that contribute to a woman's decision to have both breasts removed, few studies have actually evaluated the mental and social wellbeing after surgery among

women with sporadic breast cancer.

So this is a prospective observational study. It recruited individuals from two medical settings, the Comprehensive Cancer Center and a community clinic and they evaluated a range of mental and social wellbeing factors. Their primary outcome was cancer worry.

They also looked at other secondary outcomes such as body image, decisional regret, decisional satisfaction, and quality of life. They evaluated those outcomes pre-surgery and then post-surgery all the way up until about 18 months. So the key findings here are compared with women who kept the healthy breast, women who had both breasts removed, and as you can see here, it was about a little less than 20 percent of the cohort of 50 women chose to have both breasts removed.

Women who had both breasts removed had more cancer worry pre-surgery, and this had been documented in other studies. But what they found in this study was that over time, post-surgery, that that cancer worry did go down to about the same

level as what was reported by women who chose to keep their healthy breast. But they also found that women who remove both breasts had more concerns about body image after surgery and a lower quality of life after surgery even though they had the same quality of life before surgery as the women who decided to keep the healthy breast.

So this, although the study sample is small and the study may require replication, this would suggest that additional resources may benefit women, particularly women who are experiencing a high level of distress pre-surgery to counsel them with respect to mental and social factors that may negatively impact their wellbeing post-treatment.

Yes.

MS. HUNT: Did you compare their rates of recurrence as well? I mean --

DR. DAUGHERTY: She was not looking at rates of occurrence. This was focused on the mental and social wellbeing.

DR. ARORA: And these are three-year awards. So you know, follow-up --

DR. DAUGHERTY: The timeframe -- it's difficult to capture much information.

Okay. So this last study that I'll be highlighting is in our survivorship category and this study is focused on the question of how to optimize post-treatment surveillance. We actually have four studies that we funded around this question. Each of the studies is looking at a different cancer. We have a study on prostate, lung, colorectal, and -- prostate, lung, colorectal, and breast.

So the idea here is that with frequent post-treatment surveillance, the hope is this, that you're able to detect a recurrence at an early enough stage that you can implement curative treatment. However, not all individuals are eligible when they have their recurrence diagnosed for curative treatment. So it's really important to understand how frequently the surveillance should be offered post-treatment in order to maximize the long-term survival benefits, but also minimize the potential harms that may be occurring as a result of

repeated surveillance tests.

So this particular study by Dr. Kozower and his research team was asking the question about intensity of surveillance post-treatment for individuals who have received a lung cancer resection and whether or not increased frequency could improve survival. So the guidelines with respect to surveillance post-treatment for lung cancer patients are pretty broad. There are some organizations who suggest three month intervals and some who suggest one year intervals within the first few years post-treatment.

So this retrospective observational cohort was developed through the National Cancer Database. They were particularly interested in looking at recurrence and overall survival at five years. And so, he compared individuals who had CT scans at three months, six months, and 12 month intervals. And what he found was that compared with getting an imaging test every 12 months, receiving tests at three or six months made no difference in how soon doctors detected a recurrence and how likely

patients were to live five years after their first surgery.

This last slide, and I'm going to pass it over to Neeraj is from Dr. Kozower again, from his project monitoring report. And we felt that this was an important quote to share with you because he really describes here how engagement has helped transform for him the way that he conducts his research and how he plans to use that approach or that model in his future applications.

DR. ARORA: So you know, we heard from Sarah about finding some samples of early completed studies. So these are all part of our broad portfolio, smaller three year studies that we have funded initially. But we thought we should also let you know about some larger multisite studies that are being conducted right now all across the nation evaluating very important decision dilemmas all across the different phases of the patient's cancer journey from prevention through advanced illness care.

So an example in prevention for instance,

is a study we funded in California that's currently in enrolling young boys and girls to focus on increasing uptake of HPV vaccination among 17,000 youth who are receiving care from federally qualified health centers that typically provide care to low income families.

An example of screening, many of you saw Dr. Esserman present on the WISDOM trial at the PCORI annual meeting in our session on personalized medicine where the research team is evaluating risk-based approaches to breast cancer screening versus the one size fit all annual approach to screening mammography. And they have enrolled more than 23,000 patients to-date. An example of treatment we want to highlight is a study that we recently funded as part of our pragmatic clinical studies program where the investigators and their team are looking at comparing different approaches to treating a recurrence of bladder cancer. They are enrolling 900 patients and their caregivers.

This study was a culmination of a significant effort that the awardee team did as part

of a couple of engagement awards. We gave them where they work with the Bladder Cancer Advocacy Network to build recess capacity amongst the stakeholders and that then resulted in the successful proposals to our pragmatic clinical studies initiative. So a great example of how engagement led to a CER study.

We also wanted several studies in the area of symptoms and side effects management. On the study we want to highlight is one that we funded to Dr. Scott Ramsey where they are addressing a very important to think wisely question of what is the optimal use of colony stimulating growth factors to prevent febrile neutropenia, which as you may know, is a debilitating side effect of patients undergoing chemotherapy.

The reason we want to talk about this study because it's an excellent example of our collaboration with our colleagues at the National Cancer Institute. The research team is enrolling more than 3,000 patients from 40 different community oncology research practices all over the country

that are part of NCI's Community Oncology Research Program or NCORP. And this is one of the largest trials funded as part of the NCORP program at NCI.

An example of advance in this, we are currently conducting a study funded two investigators at Massachusetts General Hospital. It's the largest evaluation of telemedicine and palliative care. And again, here the investigators are enrolling 20 different sites that are part of Palliative Care Research Consortium that has been funded by the National Institute of Nursing Research.

While we don't have findings from these large trials, we thought we should share with you some of our early lessons that we have learned from our experience of overseeing these trials and want to highlight a couple or three different points.

One, our PIs have realized that it does take extra time and resources in conducting these large multisite trials, especially in the initial phase of the study. For example, we heard from Kristin when she was presenting on the engagement

presentation that the whole idea -- notion of communicating equipoise especially for preference sensitive decisions in a way that minimizes provider bias takes a lot of thought and time to think about in putting together a study materials.

For many of our systems studies that especially that are conducting the studies in several community oncology practices that have -- that can vary in the resources available to them. It does take a lot of time to integrate these multisystem interventions as part of routine clinical workflow.

Similarly, we realized that it takes significant coordination and relationship building between the awardee team and study sites, especially when you're enrolling patients from 20 to 40 different sites all over the country. And finally, especially for our multi-component systems intervention, it does require a very systematic plan for monitoring the implementation of interventions across different sites in order to ensure fidelity of the intervention.

Just a few summary observations from our portfolio. Our awardees are indeed engaging stakeholders to address several important decisions elements across the patient's cancer journey, right from prevention through advanced illness care. Many of our studies are emphasizing the full spectrum of outcomes that matter to patients and where possible caregivers, as well.

Many of our studies are building on prior efficacy trials that have been funded as part of many of them, part of R01s by the NCI for instance as well as studies funded by AHRQ and taking those products now to scale in real world settings and with patients were often excluded from traditional trials. And finally where possible, our awardees are trying to be efficient by leveraging existing registries and clinical trials infrastructure to address questions that are important to our stakeholders.

As I, you know, indicated that we realized that conducting such the large multisite trials that are currently ongoing in real world practice

settings does require a significant amount of time and planning and resources. And we know that there have been some discussions amongst our leadership and the Board of considering a planning or pilot phase of these studies to ascertain their feasibility and then potentially probably we should fund their actual CER trials.

We also realize that while many of our therapeutic trials that we fund for five years can indeed evaluate the impact of the interventions on patient-centered or patient reported outcomes. But if we really also are interested in looking at clinical outcomes at death or recurrence of the illness or diagnosis of a second primary or late effects such as cardiotoxicities, we may want to fund out some of our promising clinical studies for longer follow-ups that could maybe as a result we might want to partner with other funders or consider supplemental funding down the road.

Then I'm going to pass it on to Sarah to explore some future directions and then wrap up our presentation.

DR. DAUGHERTY: So as you can see, we have a rich portfolio of various cancer studies, both observational and clinical trials across the patient cancer journey, many of which have been driven by investigators partnering with stakeholders to address key decisional dilemmas that matter to patients and the people who care for them.

We have recently posted if you've seen the Cycle 3, 2019 Broad PFA, we've recently posted some research areas of interest. Two of the four research areas of interest were related to cancer and we anticipate the applications related to these particular research areas of interest coming in January, 2020.

So it's at this time that we would like to transition our discussion with you all to hear from you about topics or other areas of interest that you think we should be considering, future processes that we should be considering for our cancer portfolio. Neeraj and I have started a list and would like your feedback on this list and of course other ideas.

Based on our review of our portfolio, we noticed that we have very few studies on diagnostic intervention. We also thought that it might be important to consider novel treatments in real world settings. As Neeraj had mentioned earlier, one of our strengths is that we encourage our investigators to consider real world settings and include patients that are often excluded from other trials or studies. We're aware of a growing segment of the population including post-treatment cancer survivors and their caregivers and older cancer patients with multiple chronic conditions.

And this last bullet is with respect to cancers with increasing incidents. There was an interesting article in *Lancet Public Health* earlier this year that showed steeper rises in incidents increased for successively younger generations. And this was for six of 12 obesity-related cancers that they looked at. This included cancers like multiple myeloma, gallbladder, kidney, and pancreatic cancer. And those are all cancers that we have not highlighted independently in our portfolio and

because of their changing pattern and incidents might be worth considering in the future.

So before I end and look to you all for guidance on where we should go next, I do want to acknowledge all of the PCORI staff that has helped us put together this presentation. They have pulled the data from our various databases. In particular, I'd like to recognize Emily Lazowick who's here in the room today and Sindhura Gummi, who as program associates provided us with tremendous support and have helped create some of the slides that we presented to you today.

So thank you very much and we look forward to our discussion.

CHAIRPERSON GOERTZ: Great. Thank you so much for this very informative presentation.

Larry, do you want to start off our discussion?

MR. BECKER: That was really terrific on to your question about a list. So you mentioned that the surviving people who are survivors of cancer is

growing and growing. I happen to be on of those.

The question that's always been in my mind is I went through a whole bunch of treatments and scans and all that other stuff. It'd be interesting to understand what the impact of those things are relative to future cancers, you know, is treatment worth the cure so to speak? And what's the impact?

DR. GOODMAN: So we've discussed here a lot our ability and sometimes our suspicions that we're not able to attract the very best applications. And you have very a heavyweight competitor with NCI. What insight do you have or what studies have you done to try to understand both the awareness of PCORI among NCI investigators -- funded investigators, and whether there are things that we should be funding that we're just not getting proposals for or you'd like to fund and how are you responding to that?

DR. ARORA: So first of all, I would say that I don't consider NCI to be a competitor. So, you know, we are doing work that's complimenting each other, I hope. So just for background, I'd

like to let you know that in 2016, we had created a report of our portfolio that we had funded in cancer in our early days. And we wanted to share it with the leadership at NCI to make sure that what we were doing was complimentary to what they were doing.

And we got really good feedback from Dr. Croyle who was the Division Director of the Division of Cancer Control and Population Sciences saying that indeed PCORI was filling a unique space in the research continuum where we were taking a lot of the promising interventions that were funded as part of R01s. Establishing that efficacy and then translating them to evaluating them in real world settings. And a lot of the topics at that time that we were looking at were not being funded by them.

And now in terms of investigators, clearly there's an overlap. There's a significant overlap in the investigators. And in my mind it should be because NCI and American Cancer Society and AHRQ have created that pool of researchers that, you know, really ready for comparative effectiveness research, the type of which we focus on.

So I think our work is very complimentary to them. We could definitely do better moving forward. And that's where we need guidance from all of you as a about how we can be more synergistic with what other funders are funding in terms of topics like Sarah started, you know, a list of areas that we would like to see more studies happening.

I'll give you an example. I was part of the planning committee for a workshop that the National Academy of Medicine did. And the conclusion of that meeting was that there's a big realization now on the needs of long-term cancer survivors. But we haven't done much in terms of addressing those needs with competitive interventions like type of which, you know, like Larry was just raising.

So there are several areas we would wish we would get proposals, but that's where we want guidance from all of you as well. I hope that that's answers --

CHAIRPERSON GOERTZ: Josie --

DR. BRIGGS: Oh, sorry. Just quickly in

response to your question Steve, it is correct that NCI funds a lot of patient-oriented research, probably more than most of the other NIH institutes. But that may be part of the success of this portfolio is that there are more people ready for this kind of PCORI-centered research that I think does distinguish itself from the primary focus of NCI. It may be easier to do this when there's a large and effective institutional investing in comparable areas.

DR. DAUGHERTY: I just wanted to add on we have a working group called the direct comparisons of clinical options working group. It's actually been led by Christopher Friese and a few of PCORI staff. And we are exactly looking at that. What are some of the strategies for how we can reach additional investigators in areas that we're very interested in. And our working group is coming up with a series of recommendations we'll be presenting to the SSC in the future.

So it is an area that we're actively working on and considering in collaboration with the

Board members.

CHAIRPERSON GOERTZ: Great. That's great to hear. Alicia.

DR. FERNANDEZ: Thank you. I think Steve misspoke, but I think that he is getting at something that is crucially important, which is about where it -- in so much as that we're not competitors at all with NCI, but I think that he's getting up something that's crucially important that you also are raising, which is where are the things that NCI does and what are the things that we do? How do we move this forward to mold most benefit patients throughout the country.

And to that end, I want to suggest a few things. One is very rapidly that we should think about ways in which we can leverage PCORnet to answer those sort of questions that Larry, for example, just asked. This is a perfect observational question for which like PCORnet could be put to use. And there's no reason that it, to my knowledge, that we couldn't have a series of announcements in cancer which were specifically

geared towards PCORnet.

The second is that I have full faith in NCI's ability to test one cancer regimen against another. And I find those types of questions personally less interesting for PCORI though extraordinarily interesting for patient care. And what I think is more to my taste around what we are doing are things that such as, for example, we know that the average mean, and you brought this up the mean screening rate for colorectal cancer is somewhere between what -- 60 percent of the eligible population gets screened with many subgroups getting screened at much lower rates.

The history of mammography has shown us that a determined patient, a determined movement, determined of focus can get that rate up and choosing something such as colorectal cancer screening and really trying to really move the needle through a series of studies so that that can get to a better place for both the population at-large and for populations that are even with below -- even below that abysmal rate could be an

incredibly important thing that the PCORI can do.

And that I would ask you not to say what is missing from the portfolio because that implies they go shallow, go broad. And instead ask the question, where is the science ready for impact and where can the PCORI make a dedicated impact?

And I'll shut up in a minute, but the second issue, health services issue related to cancer screening that I would like to see us do, is figure out how palliative care can be most integrated into routine cancer therapy. And that is an area where the science is now there. It is unbelievably patient-centered. People want to feel as best as they can during their treatment and while they're living and dying with their cancer. And yet it is not something that is readily available throughout the United States.

What I love that you had a telehealth one. What worked? What's the next study on that? And what is the next study on that? So again, I urge you to think about, go deep, move the needle by going deep and let NCI work out the genomics of the

testings and work out which cancer treatment -- at which dosage of which drug is better for whom. They will do it really well.

DR. ARORA: Alicia, I would just say that as a long-term cancer survivor and one who's leading PCORI's palliative care initiatives, what you said is music to my ears. So thank you.

CHAIRPERSON GOERTZ: Thank you. Gail.

MS. HUNT: Yeah, I'm glad that you brought that up Alicia, because I was going to mention that I know Neeraj has a portfolio of palliative care and I'd love to hear, to have some kind of presentation on that for the Board about that palliative care issue. And specifically, I know that there are not enough palliative care docs, but also there has to be better ways to deal with patients in the home. So it can't just be you go to the hospital and that's when you see the palliative care doc and that's it. We've got to find some new ways for that. So I second what you said.

But the other thing is I was really glad to see this, at least a quarter of these study deals

with the family caregiver and those issues. But I think we did actually come up with some out-of-pocket costs for those people. It's not just their quality of life, you know, not typically in their quality of life, but as such as quality of life. It's the caregiver may be having to quit a job to, you know, there's a cost associated to that, that I think that's into what the PCORI is entitled to do.

So there are out-of-pocket costs for the caregiver, particularly when it's treatment time. But then it would be interesting to look at what are the costs to the caregiver over time survivorship, because we know that that's the case, too. So with the time that they take off and the impact of money to the family, out-of-pocket costs for the family. So that would be great.

CHAIRPERSON GOERTZ: Thanks, Gail. Bob.

DR. ZWOLAK: Thank you. Those are very nice presentations. I have a question for Neeraj and a question for Sarah.

So Neeraj, it struck me that of the $313 million that we've awarded for cancer research, one

study for less than a million dollars, less than third of one percent of all the awarded funds went to a PCORnet-based study. And that must be across all of our research an enormous statistical outlier on the negative side. Why -- have you analyzed why so little of cancer research has been posted or awarded to PCORnet groups and going forward, assuming we believe that PCORnet can perform research better, faster, and cheaper, as Josie mentioned that in her introductory comments. How do we resolve that? Is there a reason that our cancer studies didn't go to PCORnet?

And do you want me to stop there? Maybe I should stop there and let you do that.

DR. ARORA: You know, I'll probably invite others who are more familiar with PCORnet initiative to answer that. But that is true that you know, we've not had, I know that there's a cancer interest group within PCORnet that has a little bit of seed money to evaluate different you know databases that can be used for looking at cancer studies. But we haven't leveraged PCORnet for cancer studies. I

mean, that's the reality now and I know that you all are going to have a focused discussion on PCORnet sometime today. So maybe that might worth thinking through how we can leverage PCORnet for cancer.

But clearly, you know, we are talking about uptake of different interventions, modern treatments for instance. How and -- what is the uptake in the real world settings PCORnet can be leveraged to study those. Alicia mentioned that as well.

So I think PCORnet is primed to answer several questions, but we haven't funded it and I don't have a specific answer to why that is the case that has not happened.

CHAIRPERSON GOERTZ: Josie wanted to make a comment.

DR. BRIGGS: So I want to thank both Alicia and Bob for that comment. And I hope that our process of discussion on PCORnet priorities, both in the planning session today in the meeting with the RTC and SOC jointly tomorrow, we are able to come back to the entire Board with an answer or a plan moving forward that better addresses it.

I do think it will be an important part of that to understand what piece -- so there's been issues raised around what kind of observational studies and when is PCORnet the infrastructure to do observational work. And also -- and Larry raised some important observational kinds of questions that that might be very efficiently answered through this network.

And then there's the question around interventional studies and when is it the right framework for that? And I do think the Board collectively is going to have to challenge the -- to struggle with those issues moving forward. Some of those questions, there are well-funded NCI resources to study.

So PCORI has to be strategic in thinking about how best to use its resources moving forward. But I'm delighted. I do think PCORnet has potential that has not yet fully utilized. And so, I'm delighted to be interested in pushing this forward.

DR. ZWOLAK: Thank you. And thanks very much. The question I have for Sarah is we -- for

traditional outcomes, we talk enthusiastically about heterogeneity of treatment effect and how statistics can roll up and make important issues disappear. And regarding the study of breast cancer I worry that, that there's this statistical roll up effect for people with dramatically diverse opinions. And so for instance, it's absolutely clear, at least in my practice over the years that women who have seen a mother, grandmother, sister die of breast cancer are potentially much more predisposed to having a bilateral mastectomy.

And could you address -- and I read the -- I went onto the PCORI website and read the abstract of this study and then also the scientific presentation of this study. And it really, I admit to not having read the entire study or the discussion thereof, but I worry that in these studies about preferences that the heterogeneity of individual people's preferences get lost.

DR. DAUGHERTY: I think that's true. And I think there's an acknowledgement that there are a variety of factors that impact a woman's decision to

remove both breasts. I do think that this group tried to narrow their population to those that had non-familial breast cancer. So anybody that had, I think it was more than one first degree relative was not allowed to participate in the study. So there was some attempt to try and reduce some of the heterogeneity in terms of family history that may have potentially influenced both their genetic risk, but also their perceptions with respect to their own risk for a second breast cancer.

DR. BRIGGS: This study -- whether to present this study was extensively discussed internally. It is a very small study and it's an area that very few other funders have taken on, this impact of preferences. So I thought it would be of interest for you all to hear, but we definitely concur that this study is not yet one that leads to simple advice to patients, but it's an important topic. And one I think women all gravitate to. So I thought it was worth talking about in spite of the limitations that you bring up.

CHAIRPERSON GOERTZ: Okay. Thank you.

Sharon.

DR. LEVINE: Thank you. That was a terrific presentation. I had a couple of questions of you. You made the point that I think we're all aware of the two-thirds of the cancers occur in older adults. And I guess the question of whether Medicare's policy on paying for patients is impacting the ability to do studies.

Is that one of the factors that's impacting the ability to enroll large numbers of older adults in these studies? And the question I don't have -- know the answer to is, is this true across both Medicare, traditional Medicare and Medicare Advantage or is it only in traditional Medicare?

DR. ARORA: In terms of the ability to enroll the patients? The few studies that we've been part of, we have not seen that. So, but we can definitely explore with our investigators with our payment reimbursement -- we do see that on telehealth health studies for sure. Because you reimbursement is an issue, but I wonder if my other colleagues have any insights into that.

DR. BRIGGS: Jean might have the most insight into the impact of -- this deemed entity issue.

MS. SLUTSKY: So one of the things that makes PCORI different than other federal funding agencies is when the clinical trials policy was developed at Medicare. PCORI didn't exist and so we're not what's called a deemed entity. We're hoping that there will be a legislative fix to that which would allow Medicare to pay for their beneficiaries in our clinical studies.

UNIDENTIFIED SPEAKER: Is that just traditional Medicare?

MS. SLUTSKY: it can be both traditional Medicare and Medicare Advantage. Medicare Advantage, as you may know, has a new constellation of CMS approved activities that aren't traditionally covered under the fee-for-service Medicare, specifically around social determinants of health and other things.

DR. LEVINE: And then the other was around there. In addition to younger people, the

increasing incidents of cancer in younger people. There's a growing population of people with multiple primaries and whether or not this has been an area of study at PCORI, I mean these are obviously studies that would have to occur over a longer period of time. These don't appear within 18 months, but it is a population of growing interest. People who for whatever reason and understanding that whether these are initial primary treatment related or genetics.

DR. ARORA: So we haven't really, we don't have any -- sorry, I was just looking at that and then [inaudible] that's a multiple of these. So if you could be a cancer survivor post-treatment, often Non-Hodgkin Lymphoma and go ahead and you end up having breast cancer for instance. Or you could have multiple primaries co-occurring at the same time within a short span of time, as well. So that's a very good input for us to explore further as to, you know, what is it really in terms of comparative effectiveness research for that population.

DR. LEVINE: And then just one final comment. There are all kinds of cancer registries around the country. Do you have -- and then there's the National Cancer Registry, do you have a sense of the comparability of the information in the range of registries, state-based, institution-based, and how easy it is to do research across the registries if registries is the source of information? Do you have any thoughts on that?

DR. ARORA: So at least I know from -- coming from NCI to PCORI, the SEER registries are the most robust cancer registries because of the amount of investment NCI makes in curating those data. So and then the linkages that have been made to the SEER registries with CMS-Medicare claims and others really to facilitate evaluating a lot of issues using those registries.

So they tend to be the gold standard compared to other registries.

DR. DAUGHERTY: Just one additional comment, for instance, with the Penson study. And I think some other studies as well. But for example,

with the Penson study, they did bring on the Capture Database, which is supported by a network of community urological clinics. And the reason they did that was because they felt that that was where many of the novel treatments were being implemented. Particularly active surveillance and some of the more modern treatments.

So I think in order to capture and bring in their sample size around those options they needed to supplement the SEER registry.

* CHAIRPERSON GOERTZ: We had a comment period scheduled for 12:30. However we do not -- we have no one present or waiting in line so we'll not be initiating our public comment period. Just to reminder that we always recommend feedback at info@PCORI.org or through our website, www.PCORI. org.

This does give us an opportunity to continue our discussion. And Michelle, you're up next.

DR. McMURRY-HEATH: Thank you. I also enjoyed the presentation. There was a thread or a flavor running through your discussion of patient

preference that made the preferences seem very pliable. Like if you just provided more information, you could change the patient preference. And I think you have to be a little bit careful about that because you're not taking into account what the preference is based upon or whether or not it's something that's even changeable.

And in addition, I think patient preference always has to be considered in balance with actual risk. And I think both in the lung cancer study where you talked about the interval of follow-up screening and in the breast cancer study, it was not balanced enough against the actual risk. For example, the lung cancer patients were not stratified by the staging of their lung cancer. Perhaps their clinicians had very clear reasons or their subtype. So you just want to make sure you're taking those elements into account.

DR. DAUGHERTY: I would completely agree. And just as a reference, Kozower did publish a second study on stage one lung cancer patients within his larger cohort. So you may might take a

look at that as well.

CHAIRPERSON GOERTZ: Okay. Mike.

H: Just some thoughts on cancer and kind of the fundamental question of what we need to be looking at. I think payers are very interested in gene therapy for resistant cancers. And what the expectation is for the future gene therapy with cancers. As you all know, they're extremely expensive therapies in a very difficult to treat population.

So and I'm sure there's all sorts of studies going to be going on regarding gene therapy, but just we don't want to be left behind or we don't want to duplicate that from a payer perspective that would be supremely important, I think, because of the costs and just difficulty of the entire decision-making around the refractory cancer patient.

CHAIRPERSON GOERTZ: Okay. Thank you David.

DR. MYERS: Thank you both for fascinating presentation. It struck me that if the fates align

and PCORI is reauthorized with new language about the ability to understand costs, that that's an area that PCORI will have a unique place to do. And that from the patient perspective and the family perspective, and I think Gail you started this, that future studies trying to understand the implications of the totality of implications from a family perspective and cost will be interesting to payers as well as patients. So it's an important area to think about in PCORI 2.0.

And I had another point, but I think I'll do it offline.

DR. DAUGHERTY: I just wanted to comment actually on both of your comments. First with regard to cost, we do have a few trials that are looking at costs. I know in particular one that I oversee on DCIS comparing guideline concordant care with active surveillance and financial burden is one of the outcomes that’s looking at as secondary outcome.

And then I also just wanted to mention to your point with regard to genetics and making sure

that the PCORI is not falling behind. Just as a reminder, I think on that last slide that I showed you, we did post two research areas of interest, one of which was trying to address the evidence gaps with respect to gene sequencing and how it may influence therapy. So there were I think three or four different suggestions for head-to-head comparisons, considering companion diagnostics and genetic algorithms and so on. So that's something that we are actively pursuing as well.

* CHAIRPERSON GOERTZ: Great. Thank you. Well, thanks both Neeraj and Sarah just for a great presentation and a very important discussion and we look forward to working with all of you guys as we look at the PCORI 2.0 and what that means for our cancer portfolio.

Before we adjourn, I'm going to turn the mic over to Josie for some closing comments.

DR. ARORA: I just wanted to thank all of you for your input. And like I said, hopefully this was first of many conversations that we have to give you a guidance as we move forward. Thanks.

[Applause.]

DR. BRIGGS: I just want to add my thanks to such an engaged Board posing thoughtful questions I think will help engage staff in preparing for PCORI 2.0, which we're quite confident is going to happen.

CHAIRPERSON GOERTZ: Thanks. So I'm going to close then by again thanking those who joined us today and either in-person or via webinar or teleconference. A reminder that all materials presented to the Board today will be available on our website. Today's webinar was recorded in our archive will be posted within a week or so. As always, we welcome your feedback at info@PCORI.org or through our website. Thank you for joining us and have a wonderful afternoon.

[Whereupon, at 12:41 p.m., the Board of Governors meeting was adjourned.]