PCORI Webinar Series: Considering the Full Range of Outcomes in PCORI Research -- Payers, Purchasers, Providers, Health Systems and Pharmaceutical Industry

Event Date and Time:
Tuesday, October 6, 2020 - 02:30 pm to 04:00 pm EDT

Opening

[00:04-01:55]

- **Adam Bloom-Paicopolos, Public Policy Associate, PCORI:** Good afternoon, everyone, and welcome to today's webinar considering the full range of Outcomes Data in PCORI research, Payers, Purchasers, Providers, Health Systems, in the Pharmaceutical Industry. Before we get started, I'd like to go over a few items, so you know how to participate in today's event. Next slide.
- We've taken a screenshot of an example of the attendee interface. You should see something that looks like this on your own computer desktop in the upper right corner. You're listening in using your computer's speaker system by default. If you would prefer to join over the phone, just select telephone in the audio pane, and the dial in information will be displayed. You will have the opportunity to submit text questions to today's presenter. Your questions into the questions pane of the control panel. You may send in your questions at any time during the presentation. We will collect these, and our moderator for today's event will address as many as possible, in the allotted time during the Q&A session towards the end of the webinar. Next slide.
- Please note that this webinar is being recorded, and all submitted questions will be part of PCORI's public comment collection on our proposed principles for the consideration for the full range of outcomes data. The recording and webinar transcription will be posted after this event on PCORI's website. Additionally, you can access the proposed principles and provide comments on our website as well. Here's the agenda for today, for this afternoon. If you have any technical difficulties during the webinar, please contact our AV team AVSupport@pcori.org. I would now like to turn it over to PCORI's Executive Director, Dr. Nakela Cook.

Welcome

[01:55-06:18]

- **Dr Nakela Cook, Executive Director, PCORI:** Good afternoon, everyone, and welcome to the second of two webinars as part of our series on the consideration of the full range of outcomes data in PCORI Research. I'm Nakela Cook, Executive Director of the Patient Centered Outcomes Research Institute, and I'm excited today to kick off the discussion on a topic that I know is deeply important to you and will be central to PCORI's work over the next 10 years. And that is how we will approach our new provision to take into account cost burden, and economic impact data as part of our funded research. Congress' reauthorization of PCORI funding for another 10 years, gave us all a remarkable opportunity to continue to fund research that matters to you, and that will help to advance patient centered, efficient, and effective healthcare systems. The reauthorization language encourage us that our work will be even more relevant and useful to our stakeholders, by allowing the consideration of the full range of clinical and patient centered

outcomes that meet the needs of patients, clinicians, purchasers, and policymakers. This includes the appropriate capturing of potential burdens and economic impacts of various health care services, along with the relative health outcomes and clinical effectiveness measures our work has captured to date. As always, we embrace our role as the nation's premier funder of clinical comparative effectiveness research, and patient centered outcomes research, with a deep commitment to multi-stakeholder input. Today is one of the many activities we will undertake to understand the diverse perspectives on health care costs burdens, and economic impacts. We know that there are going to be differing perspectives on these issues, all of which, though, we desire to hear as we strive to take a balanced approach forward. As is true for all the work PCORI does, we're beginning with engagement. We want to hear from you as partners and key stakeholders in our work. And at this initial step of our implementation efforts, your input and expertise will guide PCORI as we implement this provision and develop guidance for future applicants of PCORI's funded research. We're taking a very broad and deliberate approach to implementing this new authority, one that will consider principles to guide our research, updates to our methodology standards, and discussions about how PCORI can contribute to conversations about health care cost and value. And as part of this approach, last month PCORI's board of Governors approved the release of a set of proposed principles for the consideration of the full range of outcomes for public comment.

- These principles are a high level framework describing PCORI's interpretation of this new legislation, and following the incorporation of input from sessions such as these and public comment, these principles will serve for us as a basis for PCORI as we develop guidance for future applicants. For today's discussion, we're going to focus on these principles, hearing from an excellent panel individuals, representing the perspectives of payers, purchasers, providers, health systems, and the pharmaceutical industry. They will share their thoughts on what cost burden and economic impact data to capture, opportunities and challenges on how such data can be captured, and when it is appropriate to capture this data. This will not be the only opportunity we hear from the public and our stakeholders on this topic. We hope that you provide your feedback during the open public comment period on the proposed principles on our website, and we're excited to hear from this panel, but also from all of you. As we move forward with our implementation efforts, we will ensure that in all the work we do, we remain true to our overarching mandate to generate authoritative clinical evidence that will help patients, clinicians, purchasers, and policymakers in making informed health decisions.
- Thanks again for taking the time to engage with us on this important topic today, and thank you to our moderator and panelists for lending their time and expertise to this discussion. And with that, I'll turn it over to Andrew Hu, our director of Public Policy and Government Relations at PCORI. Andrew.

How We Got Here

[06:18-14:11]

- **Andrew Hu, Director, Public Policy and Government Relations, PCORI:** Thank you, Doctor Cook, for the introduction and for the opening remarks. Good afternoon, everybody. I'm Andrew Hu, Director for Public Policy and Government Relations here at PCORI. Before jumping into the presentation on how PCORI got to this point, I want to echo Dr. Cook, and thank everyone for joining us for today's discussion. Next slide.
- So, before getting further into the discussion, it's always helpful to know what's in the law. Included in our Reauthorizing law that was passed and enacted at the end of last year, is new authority for PCORI to capture, as appropriate, the full range of outcomes data in the course of our research studies. The supervision expands the range of outcomes data that PCORI

has already been collecting to now include cost burden and economic impacts related to the utilization of health care services, and also a collection of more patient centric perspectives on cost and burden, as well. This includes medical out-of-pocket costs, including health plan, benefit, and formulary design; non-medical costs important to patients and families such as caregiver burden; effects on future cost of care; workplace productivity and absenteeism; and costs associated with healthcare utilization. Next slide.

- Getting to this final policy took a lot of the cooperation and a lot of input from the broader healthcare stakeholder community, Congress, when debating causes your authorization last year, her from a wide range of stakeholders on this topic. Understandably, the topic of trusting cost and value continues to be a sensitive subjects among the broader community as it was when PCORI was first authorized and established 10 years ago. So to make sure that there was a balance between those who had advocated for PCORI to conduct cost effectiveness analysis research, and those who believe the core's research should remain fairly clinical, Congress led it on a compromise to include this new mandate for PCORI to consider a broader range of outcomes to now include costs and economic burdens, but maintained prior prohibitions on cost effectiveness and the establishment of quality adjusted life year (QALY) thresholds. Next slide.
- So while we'll be discussing PCORI's proposed principles for the consideration of the full range of outcomes today, and this webinar and public comment period on those principles is just one part of the broader implementation plan, given the complexity of this topic, PCORI is proposing to take a very deliberate and transparent approach to implementation, and we've broken this into three separate pillars. The first, pillar one, is meant to serve two separate, but related goals. The first, as Dr. Cook mentioned, is the establishment of high level principles, which will inform the public on how PCORI is interpreting this new provision. And those for post principles were approved by PCORI's Board of Governors to, to be released for public comment last month. The second is, based on those principles and the input we receive on them, is to develop guidance for our future applicants on how they can actually operationalize this new authority into their research proposals. We're hoping to finalize the principles and develop guidance for our applicants by February or March of next year.
- Pillar two is to establish an update methodology standards to further inform how PCORI funded studies should capture this data. PCORI's methodology committee will likely play a leading role in this effort, and we expect this process to remain transparent, but to take a little bit longer, in terms of timing with the ultimate results being updates to PCORI's methodology standards.
- And pillar three is a discussion focused on precarious role and how this information can and should be used. We expect that this discussion will be ongoing as it relates to broader policy and clinical decisions.
- What's important to note is that these three pillars do not have to happen sequentially, and we are already working through each bucket to identify opportunities for PCORI to play an impactful role. But for this webinar series and today's discussion, we are focusing on pillar one and getting input from the broader stakeholder community on this new authority, and to and to provide input on the proposed principles. Next slide.
- So, in terms of progress, as I mentioned, we're still focused on pillar one right now. Included in this, pillar is the release of the principles, which, as you mentioned, PCORI's board did last meet at their last board meeting in September 14. That opened public comment period, which, as you can see, we're squarely in the seeking public input phase of this process. That 60 day public comment period is now open and will close on Friday, November 13th. Additionally, this webinar series, and the input that we receive from it, will feed into that public comment record

that we will keep. From there, we will take the input received from the public comment and from these webinars, and revise our principles and develop guidance for our applicants. As I mentioned, our target date is the final principles approved by PCORI's Board of Governors, by February or March of next year, and we expect that guidance to be developed for applicants ready for the Spring 2021 funding cycle. Next slide.

- Getting to the principles: this slide is meant to highlight what they are, why we need them, and how we expect them to be used.
- In terms of "what," these principles are high level framework to describe PCORI's interpretation of this new mandate. In terms of "why" we wanted to develop them, we felt it was important for PCORI to provide the public and future applicants with an understanding of our interpretation of this mandate. And lastly on "how" they will be used is, as I mentioned, we expect these principles to serve as a point of reference for PCORI as a basis for our development of future guidance to potential applicants, and for updating of course methodology standards. These principles themselves are not meant to be the standards and methods for research. Next slide.
- Getting to the principles themselves. As you can see on the slide, we've listed the four principles that PCORI has proposed. The full document itself is up on PCORI's website, long with a form where you can submit any comments you may have on them. On the left-hand column, you'll note that principles 1 and 2, focused on identifying the relevant cost burden, and economic impact, outcomes and measures. Principle one notes that PCORI-funded research, may consider the full range of outcomes important to patients and caregivers, and this includes those on the burdens and economic impacts.
- Principle two directs our research to consider the outcomes important to respect the stakeholders when those outcomes have a near term or long term impact on patients.
- The column on the right, principle three, describes certain criteria regarding the collection of data. Principle three notes that the collection of data on burdens, and upcoming impacts of treatment options must be appropriate and relevant to the clinical aims of the study.
- And below that, principle four opens a discussion about the possible consideration of certain economic analyzes. We acknowledge in this principle that beyond the collection of data itself, PCORI may support and conduct certain types of economic analyzes as part of a funded study to enhance the relevance and value of the information to healthcare decision makers. Next slide.
- So here again, are the references and resources for you. These links will take you to the proposed principles, as well as the public comment form. And we do hope that we receive input through this discussion, the public comment, as well as any other assessment may have as we move forward in our implementation process. And with that, I will now turn it over to our moderator, Anand Parekh, who is the Chief Medical Officer at the Bipartisan Policy Center, to begin the discussion. Anand, the floor is yours.

Panel Discussion

[14:11-1:18:59]

- **Moderator, Anand Parekh, Chief Medical Officer at the Bipartisan Policy Center:** Andrew, thanks so much for that background and introduction. Congratulations to PCORI in really, I think, putting forward a process that is open and transparent and invites public comments. I think this is exactly how it should be done. And I'm honored to join you with a distinguished set of panelists today. You know, this topic, I think, just to underscore, is so important. In this country, as all of you know, we spend about three point eight trillion dollars on health. And unfortunately, about a quarter of that expenditure, studies have shown, don't really go to

improve health outcomes. We've also heard from Dr. Cook, that as we move from fee for service to alternative payment models of value based health care transformation is so important that is being advanced in our healthcare sector. And as we know, value has two components to it: it's the quality and the outcomes piece, as well as the cost piece. So, this is, therefore, pretty important. And then I think, most importantly, we're all here because we want to empower individuals, patients, and consumers, and families. And individuals need a full suite of information, and they need information on outcomes, but we're also hearing they need information on costs: that's really important with respect to shared decision making. So, I think, for all of these reasons, I think it's an important conversation to have, it's important to bring stakeholders together to do this right. So the how part is really important.

- As Andrew mentioned, over the next, about 45 minutes or so, we're going to be discussing the what, the how, and the when. What are the key data that ought to be collected as PCORI goes about fulfilling its new mandate as identified in the reauthorization? So the “what” part. The “how”: how do they go about doing that? And then the “when”: when is it appropriate to take into consideration, or collect information, related to costs as well as the economic impact?
- I want to first start by introducing the distinguished panel, and then we'll go through a couple of rounds of questions. And then I think we want to make this interactive, and so, hopefully, we'll have some questions from the audience. So we have, as panelists today, I'm really pleased that we have Randy Burkholder joining us. Randy is the Vice President for Policy at PhRMA. And in this position, Randy leads work on policy solutions for supporting continued biopharmaceutical innovation in high quality patient centered health care, including payment and delivery reform, quality measurement, appropriate use and patient adherence, evidence based medicine and health technology assessment, as well as issues related to the value of innovation and personalized medicine. Welcome, Randy.
- We also have Shawn Martin with us. Shawn is the Executive Vice President and the Chief Executive Officer of the American Academy of Family Physicians. In this position, Shawn provides the AAFP Board of Directors leadership and advice, and he works with them on the mission, strategy, and vision of the organization. And he also provides representation to organizations in the medical and public health and private sectors.
- We're also very happy to have Beth Monsrud join us today. Beth is the Senior Vice President and Chief Financial Officer of UCare. UCare as a not-for-profit health plan in the Midwest. Beth has more than 20 years of experience in financial management, she serves as the corporate treasurer. Her responsibilities include strategic direction for Ucare’s accounting, healthcare economics, reporting systems, budgeting, forecasting, and other financial duties.
- We're very happy to have Margaret, I believe Margaret is with us. Margaret Rehayem: Margaret is the Vice President at the National Alliance for Health Care Purchaser Coalitions. She provides leadership there for national initiatives that support member collaboration, helping coalitions leverage the regional efforts at the national level in order to drive innovation, health, and value for community organizations across the nation. Margaret, welcome.
- And finally, we have Dr. Umscheid, Craig Umscheid, who's the Associate Professor of Medicine, Chief Quality and Innovation Officer, Vice President for Healthcare Delivery and Science, and Director of the Center for Health Care Delivery, Science, and Innovation at the University of Chicago, Department of Medicine. Dr. Umscheid is a clinical epidemiologist, as well as a general internist, and he has been funded by PCORI, as well as many others, including the CDC, the NIH, and AHRQ. Welcome Dr. Umscheid.
- So I think we have a terrific set of panelists diverse, in terms of their perspectives, and I think that's really exactly what we want to help PCORI with these key questions, So let's just start. And

maybe the first question is for the entire group on the “what” part of this. I think, in order to ensure PCORI funded researchers, capture the data we're talking about here are the economic and cost, it would first helped to know what types of data analysis you all need in order to make the decisions you do from your various perspectives. And so first question to all of you, maybe, Randy, we'll start with you and go in the same order as introduction.

- Tell us from your perspective the “what” part here: what are the types of economic and cost data that PCORI, given this new mandate in the reauthorization, ought to be focused on, and ought to be collecting

[20:16]

- **Randy Burkholder:** Yeah, thank you for that, that question. First, I think I just want to underscore that we fully support the research that PCORI does, and that involves a wide range of outcomes, including the economic and health burden outcomes that are described in the institute's reauthorizing legislation. So we see that PCORI can continue to play a growing and positive role in informing high quality, well informed, healthcare decision making in the US. Market, and supplement a lot of the research that our member companies and a lot of other researchers do, both on clinical and economic outcomes. I think there are a wide range of outcomes PCORI could be looking at.
- I think one of the things that particularly will be important for PCORI to focus on will be the priority gaps in cost related outcomes. Which, I think, sometimes might be areas that are harder to close and in the research enterprise, and that's precisely why they have remained gaps. So I think if PCORI focuses on what is easier to do, it may end up replicating the type of cost data that's already widely available to payers and other decision makers, and it might fail to close some of the gaps in evidence that are most important to patients. So we don't have enough research yet on what the most important health burden and economic outcomes are to patients, but I think we know from the data what some of those are, and we would say, let's focus first and foremost on those outcomes, and then other stakeholders, be it biopharmaceutical manufacturers or payers or others, can figure out from there how we should be helping to close those gaps and doing research that meets those needs. So, I would just highlight a few that I would I would think it's important for us to be focused on just based on what we know from the evidence already that is important to patients. First and foremost, certainly, out-of-pocket costs, including co-pays and deductibles. That's identified in the reauthorizing legislation as Andrew noted, not only for treatments, but provider services, transportation, childcare costs, and certainly productivity and caregiver burden. Second thing, I would say is fundamentally, we would agree with some of the comments made during yesterday's panel that it's important for PCORI to take a holistic approach to looking at costs of treatment and cost of care intervention.
- And the final thing, I think it would be great to see PCORI stress in this space, along with more broadly, is just making sure that it is prioritizing research that meets the needs of people with disabilities, and that helps to close some of the persistent health disparities and gaps in health equity that we see in this country.
- **Anand Parekh:** That's great. Randy, just one quick follow up question for the audience background. You mentioned priority gap areas. Do you want to just expand on whether there are a few you're thinking about it, or just provide a couple of examples?
- **Randy Burkholder:** I think probably some of the ones I just listed.
- **Anand Parekh:** OK, great. I wanted to make sure that it wasn't sort of a separate list of questions, very helpful. Thank you Randy. Shawn?

[23:43]

- **Shawn Martin:** Great. Thanks, Anand, and thank you for the opportunity to be here today. Similar to Randy, we continue to be fully supportive of PCORI in their mission and applaud the reauthorization and the slight change in direction of their work moving forward. I'm representing a group of individuals almost solely, but devoted to primary care and kind of community based medicine, I would echo Randy's comments.
- I think the out of pocket cost, whether that be through the form of co-pays and deductibles, but also out-of-pocket costs, largely associated with out-of-benefit design, care and services that an individual may seek either through a retail or telehealth platform, but it's not necessarily part of a benefit design, would be important data features to better understand. I think, from our perspective, the presence of, and the combination of those factors is probably more important than the dollar amounts in the earliest stages of this research, just to look at what that does to shared decision making. And certainly when you think of the economic pressures of accessing various types of treatments, understanding the combination of those clustering provisions becomes important. Primary care is unique: a lot of times, they are counselors, helping patients navigate a healthcare system, helping them make determinations on health care services that they likely won't provide themselves. They're just more fulfilling a navigator or patient advocate role in decisions with the caregiver and the patient.
- I, too, would stress that, I think, looking into disparities, both based upon race, gender, but also geography would be a data set that would be critically important for PCORI to think about moving forward. So those would be my initial comments Anand, thank you.
- **Anand Parekh:** Great, Shawn, thank you. Beth?

[25:51]

- **Beth Monsrud:** Good afternoon, everyone. As Anand indicated, I am the Chief Financial Officer for UCare, representing a non-profit health plan in the state of Minnesota. We have about just over 500,000 members, and we primarily focus on Medicare and Medicaid programs, and a variety of government programs with dual eligible for the elderly and disability populations. And I think it makes our plan a little bit unique in terms of, we do no commercial group coverage. So we're very mission focused and aligned with the patient centered approach of PCORI. As I looked at, the draft principles from a health plan perspective, we too highly appreciate the expansion of the mandate. Patients make choices about their health care every day, and various stakeholders like health plans make decisions that impact that care, and the related member outcomes as a result.
- And so, we feel strongly that adding this kind of expanded set of information to PCORI's funded research will really enhance the value of this research to the healthcare system overall. As I thought about "what," I thought about it in the context of the decisions that we make as a health plan. A big part of one of those major components is already what's been referenced around how we set up our products from a benefit design and pricing perspective.
- So all of those out-of-pocket co-pays, deductibles, and co-insurance and cost sharing that are included. And I think what I would add on to what the previous panels have said is it really is about the interaction and the impact that those cost burdens create in terms of how that influences the decisions of the patients in seeking care and the quality of the care they choose.
- So, whether it's along the lines of the setting or the place of care in terms of, you know, we set co-pays many times to drive to a certain setting, to drive to primary care--zero co-pay or versus $100 ER--from managing that total cost care setting. But also things, particularly around drugs and prescriptions, and the cost sharing and design of the formulary is all impacted by, you know,

from a health plan perspective, trying to manage that total cost of care. But also, wanting to balance that with the quality of the outcomes. How does it affect medication adherence rates?

- Based on recent research, we may make different decisions about, you know, how that balances between the cost aspect versus the quality of the outcomes, and wanting to drive to those highest quality outcomes. So the benefit design is a key area.
- Also, I think about it from a patient engagement perspective as well. And, you know, gathering cost data. Many times as a health plan, we are looking for ways to increase the engagement of our members in that decision making process and helping guide that effort. And so we oftentimes offer incentives to members for getting their mammograms or, other preventative-type care or wellness types of activities. Gathering information and data around design and development of programs, and what influences that behavior in terms of not just knowledge about what's best for the health, but how those economic either benefits are burdens are barriers, and potentially impact to that level of engagement.
- We also make decisions around, obviously, care delivery options. So I think that in looking at PCORI's recent research that's been published, I think about there was a study out there on various weight loss strategies. The differences between those types of procedures, the study concluded that gastric bypass created the greatest amount of weight loss, as well as remission of type two diabetes.
- That was a prime example of a study that could have brought in the impacts of those benefits and the differences between those procedures from setting perspective—the costs of different settings—as well as the different areas of coverage and options. And also, how does it change future utilization? The fact that there was remission of diabetes in that particular outcome, measuring that impact from a net perspective relative to the outcome of the different procedures. So, those are the types of things that I would say.
- I think the last comment I would make is I think one of the challenges for PCORI in implementation is going to be defining that line between reporting the data and collecting the data of economic burden and impact that is allowed in the mandate, versus that cost effectiveness. And where does that line get drawn, and what that looks like.
- **Anand Parekh:** Thanks so much for that as so many important points there, and thank you for bringing up the wellness programs and behavioral economics. I think that's a very interesting area. Margaret. You may be on mute, Sorry.

[32:51]

- **Margaret Rehayem:** Isn't that the typical thing? Thanks, Anand, for having us on today and for PCORI for inviting us. We too, are very glad that PCORI's mandate is helping to bring forward a broadening of their research agenda—it's something that we've been very closely working on through a few of our grants that we have with PCORI. When we take a look, you know, I echo a lot of the comments that folks have already expressed here. You know, the interesting thing about being almost at the ending of the panel for one question is like, OK, what's new, what do you add? So, you know, if I do add anything at this point, we want to make sure that we're also looking at objective cost data. I know there are a lot of different types of data points that are put together with health plans, PBMs, and the other stakeholders. And employers want to make sure that they are looking at the objective side of that cost data. Also, looking at impacts of costs over time with the patient.
- It was already talked about site of care and health equity, but I just want to re-iterate those two points and then also looking at policy implication and practices--that would include leave policies or any impacts on workplace culture. I'll leave it there for right now. Thanks so much.
- **Anand Parekh:** Thanks, Margaret. Craig, your perspective from a clinician and researcher.

[34:23]

- **Craig Umscheid:** Thanks for inviting me. Can you hear me? Yes, great. I just wanted to thank PCORI for inviting me, and you're right, Anand, that I come at this from a couple of different perspectives. One is an internal medicine physician and a provider, and the other from my administrative role in the health care system as the Chief Quality and Innovation Officer. And to my understanding, PCORI, for the most part, evaluates the effectiveness, or the comparative effectiveness, of different programs. So, program A versus program B, or approach A versus approach B. I think, less commonly, this studies are looking at drug A versus drug B, or device A versus device B.
- So, from our perspective, both providers and the healthcare system, really knowing the costs that the patients bear in those different types of clinical approaches that are being compared, or there's different types of programs, is absolutely essential. It's essential for shared decision making with patients at the bedside or in the clinic.
- The other thing that I was very impressed by when reviewing the principles that PCORI set out, was that we're talking not only about costs relevant to patients, but costs relevant to healthcare organizations that are providing care to patients. So I think understanding the costs of making investments in certain approaches, whether it's approach A versus approach B, an understanding of the "bang for the buck", or the return on investment of those approaches is incredibly valuable to health care systems, and really enumerating the different costs that go into new programs that might be considered.
- And I think this is all the more relevant as the country pushes more and more toward high value care, and we experience new modes of care delivery. We saw in COVID-19, just at our own medical center, that virtual visits on the ambulatory side, went from about zero before COVID to well over 100,000 over the last few months. So the type of care that we're providing in the site of care and what it looks like is changing dramatically, and understanding the cost implications of those changes to both patients and providers is really important.
- **Anand Parekh:** That's great, thank you, Craig.
- Maybe we'll shift at the second question and focus on maybe combine two areas, the "how" and the "when". Randy's starting back with you. How could PCORI go about, and PCORI-funded research (and some of this gets into methodology), go about extracting some of the data that you all just cited that are critical to be included in studies. And when is it appropriate: is it appropriate all the time? What are the circumstances where it's most appropriate to capture some of the data elements that you think are really important in this space? And we'll go down that down the line again, Randy.

[37:33]

- **Randy Burkholder:** Right, Thank you and thanks for that question. So I guess I would, I would go back and start at PCORI's first principle, which is, start with centering on outcomes that are important to patients and caregivers (recognizing that the research may ultimately be relevant and hopefully will be irrelevant to a broader range of stakeholders). But I think thinking about how to start there and keep PCORI true to its mission, as it moves down the road of gathering research outcomes related to economic burden or cost.
- In terms of the "how," I would take time to develop a clear, explicit, transparent process for that. So I think we have a really good start here today with the two panels you've held., with the comment period that you had, but I think continuing on that, continuing to develop inputs and look at what really constitutes patient centeredness and space, and developing more of a proactive explicit agenda and plan would be job number one. There, I think, as that research gets launched, one of the things on the "how" that PCORI will need to start thinking about is

how its priorities in relation to burdens and economic outcomes are going to interact with its core agenda on comparative clinical effectiveness research, or patient centered outcomes research. For example, if we want to gather more data out of pocket costs, indirect costs to patients, that will require a different type of research to begin with, probably.

- So I think having an explicit process to sort through those kinds of questions will be really important. Last thing I would say briefly is that it will be important to do just what you were speaking to, which is gathering real-world cost data to get a handle on what the holistic costs are going to be for patients. I think we, we often see that modeling based on early estimates can certainly help and be a useful data input to inform decisions. But, those data, those estimates, can have a lot of uncertainty, and we've seen that in some of the third-party budget impact analysis that have come out based more on models. In terms of the "how," PCORI making sure it's gathering real-world data that takes into account the various direct and indirect costs that are going to relate to the particular intervention being assessed, and thinking about how those direct and indirect costs relate to the care, and the patient experiences in their care journey, would be a couple of couple of top line things that I think are important.
- **Anand Parekh:** Thanks. Shawn?

[40:33]

- **Shawn Martin:** Thank you. I think top of mind for me is really looking at where there's some gaps in care. And I think PCORI has done historically a nice job of identifying smaller questions that have larger ramifications, and I think that model will continue to be of value in my mind.
- I think, expressing my bias in primary care, we tend to want to look at lower acuity/broader application types of things, versus higher acuity/narrower applications. But I do think looking at gaps in care that may or may not have economic contributors, that impact a large range of identified patients would be kind of the "where" to start. I think how to collect that data is obviously very challenging, because of the kind of disconnect between various data sources that may or may not be available.
- But, as I said in my opening comments, I think in our mind, it's more the *presence* of certain economic limitations versus the *amount* of that's really kind of more telling, at least in the early stages of some of the research—just how the presence of cost sharing or the presence of various ranges of deductibles or co-pays would impact the types of secure decisions that are made by patients and providers. Completely biased, but.
- **Anand Parekh:** Thank you, Shawn. Beth?

[42:12]

- **Beth Monsrud:** Yeah, so I echo the same thing. I think kind of keeping the focus on the patient centeredness and I particularly support the distinction between principle one and principle two in terms of the importance to the patients while at the same time being relevant stakeholders. I think keeping that focus will be important. In terms of some of the "how" I echoes some of the data challenges in terms of providing applicants resources and connections to two data sources. And then defining what those data elements can look like and mean.
- So, being able to help, and then also, I think, putting some parameters around helping applicants figure out how the design of their research questions are going to change when you add this different dimension in. Because research has been so focused on the clinical outcomes and patient perspective, having these added dimensions of cost and the impact, I think, are gonna change the questions that are asked, and the focus of the research and dimensions. So, helping applicants and putting some processes and supports in place to help, you know, really how to frame those questions, to get at the impacts and the results that were more mass multi-faceted that we're looking for.

- **Anand Parekh:** Great. Thank you, Margaret. You might be on mute again. Sorry, Margaret.

[44:19]

- **Margaret Rehayem:** I think that'll be the continual challenge for me today. I echo what a lot of folks have said already here. I think that the real-world data is probably where we really need to go. There's been a lot of focus on modeling over the years, and therefore, a lot of different types of metrics that have come out of that, which at the end of the day don't really support the, where the questions that employers are really needing to manage. And so, I also echo what Beth just said about looking at design. So, you know, other than that, I think it will be really important for PCORI to look at different opportunities to even combine different stakeholders together and see how any of the data that comes together with those stakeholders can actually help to address issues that are happening in the system.
- **Anand Parekh:** Great. Craig?

[45:23]

- **Craig Umscheid:** Yeah, I might think of this in an implementation science context: so oftentimes we see programs being compared, and one program is more effective than another, but we don't know everything that it took to get that program up and running. Some of this may be simply sharing more details, and describing more details about what it takes to build Program A versus Program B, enumerating those potential, approximate costs and comparing those costs in the context of your comparison of the outcome. So for example, if you're comparing one program that uses community health workers in the patient's home to reduce re-admissions of patients to the hospital, versus another program that uses nurses making calls from the hospital, both of those programs are going to have different types of costs. So enumerating the cost of those two programs, and sharing the effectiveness of those programs in the context of their costs could be very helpful.
- **Anand Parekh:** And, Craig, maybe one follow up that, maybe from the researcher perspective, do you think the Health Service researcher community out there—of course, these aren't necessarily new analyzes—but how comfortable, how eager, how easy do you think it is for researchers to start, to collect sort of this new data, if you will?
- **Craig Umscheid:** That's a great question. I think, for the types of analyzes that I'm describing, where you're really enumerating and describing program costs, I think you can do environmental scans and different systematic approaches to approximate costs, and I think the more that that's expected, I think the more those methods will be developed and resources to leverage that type of data will be developed.
- That said, I do think it's important not to require cost descriptions or cost analyses in every proposal that's submitted to PCORI. And that was another principle I really appreciated when we're doing the draft. Because oftentimes, we're going to be comparing two different programs or two different approaches, where we're really not sure what works. And the main question is really about effectiveness, and cost of the programs really doesn't matter if neither are particularly effective. I do think enumeration of cost is a nice first step.
- **Anand Parekh:** Great, thank you, Craig. Randy, maybe moving back to you.
- You've talked about the types of economic and cost data that PCORI ought to focus on, in terms of collection, and maybe the outcomes. What our outcomes at PCORI, perhaps, should not be considering? Or what's the road that you think would be less fruitful for PCORI to go down?

[48:46]

- **Randy Burkholder:** Sure, thanks. I'll keep it brief on this one, and maybe if we have other folks time to answer. But I think I would agree with what others have echoed already and what PCORI is recognizing, which is that conventional cost effectiveness analysis, does continue to be

prohibited under PCORI statute. I think there's a lot of opportunity within that to deliver and research cost outcomes that matter not only to patients, but other stakeholders. The only other things I would caution against would, I think, be those I've already mentioned: not over-reliance on models for cost or economic outcomes that are grounded in very early evidence, but taking the time to generate more real-world evidence on what the actual cost impacts are would be really, really helpful for understanding both the clinical and economic impact of not only new therapies, but diagnostic tests, and treatments, and other interventions. So, that would be one. I think the other is just going back to making sure that PCORI is focusing on understanding what the outcomes are, the patients prioritize the most and doing the work to close those. I do support the comment, that not every not every study PCORI does needs to include cost outcomes—I think it's more important that a subset of those studies are effectively targeting the priorities that PCORI's identified. But I think within that space of what outcomes matter to patients, I think, drilling down to the disease level but also the subpopulation level and understanding the cost and economic factors that are important to different groups of patients; be they patients subject to health disparities, those in different geographic regions, those of different socioeconomic status. They may view something like out of pocket costs or transportation costs or total treatment costs very very differently and weight them very differently. I think PCORI could make a real contribution by gathering and making more of that type of data systematically available, not only in its own findings but making it available for use in other researchers and other analyzes.

- **Anand Parekh:** Great, thanks Randy. Shawn, before I go to you on a separate question, I did want to remind everyone in the audience you can submit questions or comments at any time through that chat box, and PCORI staff will curate them and we'll have a chance for the panelists to address your comments, as well as your questions, So please feel free, and go ahead to do that, and we'll get to your audience questions pretty soon.
- Shawn, I was actually going to ask you, going back to some comments you made about shared decision making, your perspective, your members as clinicians, you know, give us a sense of sort of the types of questions when it comes to costs and the economic impacts of various treatment modalities. What are the types of questions that they get referred from patients, and where do you think PCORI's mandate here could be most helpful to the frontline family practitioner out there?

[52:20]

- **Shawn Martin:** Yeah, I think that's a really interesting question. As Randy, was speaking, I keep coming back to, I think, at the primary care level, one of the costs is really how long the economic burden will be sustained. I think that's something that we hear often is just a one-time costs, is just a monthly cost, is just an annual cost for a prolonged period of my life, or for my child or for my parent or something like that. So I think the period of time is really important.
- I think the other one is obviously something that may fall well beyond the existing scope of PCORI, but it comes up often, which is what can I expect for this cost? If I sustain this cost over a four or five month period for this particular treatment, what is the reasonable outcome that I can anticipate? Understanding that may be out of scope, but I think it is a frequent question that comes up. I think the other thing is primary care, because it's largely community based, there's the evaluation of costs that are both directly related to health, but also the costs associated with other factors that are influencing health and the social determinants. So a lot of time patients, you know, who will engage with our members around the value of certain activities versus others. And that can range all the way from food options to, you know, exercise or other types of non-pharmaceutical or non-biological interventions. And I think equating better

understanding the economic pressures on individuals and families that aren't solely related to health care is a nexus that our members find themselves in more often than probably data collects. So, balancing rent and food and childcare, and other types of costs that may or may not influence their health care decision making is probably a common…I'm going to say common, probably not every patient, but a large number of patients are balancing those needs.

- **Anand Parekh**: That's great, thank you, Shawn.
- Beth, I wanted to go to you next, and this is a little bit back to the "how" piece, when we talk about collecting economic cost data—particularly when you go beyond out of pocket costs, total cost—a lot of this information is proprietary, and if you can instruct us a little bit more about what are the implications for the data, that future PCORI-funded researchers can collect, and recognizing the issues and generalizing costs, what are the limitations in collecting these data? So if you can just expound on those issues, that'd be great.

[55:10]

- **Beth Monsrud:** Sure. So, yes, there are definitely some challenges, particularly when it comes to study. You're likely going to have a set of participants that are covered by multiple payers, with different benefits, different cost sharing, and/or even different contractual payment rates and costs for the same set of services that are going to potentially influence results or create variables that really can't be controlled in a study of outcomes. So, as I thought about some of the ways that we can potentially address those issues, I think it was Margaret that mentioned, someone mentioned earlier, about the potential to in the design of your study, partnering with a specific health plan where you can gain direct access to that, subpopulation, or specific product where you get direct access to that data in partnership with the Health Fund. That removes some of the variation of the coverage differences and potential claims payment rate differences across payers.
- If partnering isn't an option in terms of removing that variability in benefits and contractual payment rates, you can you can potentially take a little bit different approach in terms of applying average payment rates from independent data sources against utilization. So, using in your study the specific utilization information and results from the study, but then gathering cost and data from independent data sources. So, one way to do that would be, say, working with an actuarial consultant that might have access to claims datasets by specific areas or populations.
- There's governmental datasets available. There are, in many states, even locally based claims datasets. For example, in the State of Minnesota, all the health plans submit their claims data to an all-payer claims database. So, you know, I think one of the things PCORI can really do to help researchers is to, you know, provide access and connections to where some of those datasets may reside, and how that can get incorporated into the analysis and the activity. And I think the key really is not necessarily having the specific data, but kinda normalizing the data, where actual information is not accessible. And in some ways, I think it was also mentioned, it is as you're designing a study where you need claims data, it may take us back to the principles to say, is the claims data really relevant to this particular situation? It may be a situation where the benefit information that you collect from the individual participants is more relevant than necessarily the claims data to the outcome. So I think applying those principles you can also really look closely about is, is claims data really necessary?
- **Anand Parekh:** Great. Thank you, Beth. Margaret, I wanted to turn to you: as Andrew mentioned earlier, PCORI has new legislative mandate considers the full range of outcomes, and it specifically identifies workplace productivity and absenteeism as an outcome measure. And I thought, if you could just, maybe, take a minute or two to describe, how do employers define

these terms? And, I think that's important so that PCORI-funded researchers in the future ask the right question, and generate evidence that's meaningful and impactful the employer. So, want to just provide your perspective on that?

[59:35]

- **Margaret Rehayem:** OK, not even gonna say it. All right, so the workplace. When you take a look at workplace productivity, productivity itself seems a little subjective in nature. And I think employers have had a hard time really wrapping their arms around defining it. You know, at the same time, the ability—because it really is measuring beyond how many widgets are happening, you know, whether or not somebody's president work or whether or not business outcomes are being achieved, or there's a whole host of different business performance measures or KPIs that employers have relied on.
- When we take a look at, from an organizational perspective, you do want to take a look at environment, how policies and practices actually play into how the environment and the culture has brought together. Employers I know have been very interested in employee engagement and the ability for any type of impact and data that they can get around engaging whether they're putting programs in place, or they're looking at the impact of culture, both with the implementation of a program or the continuation of a program over time. So, when we take a look at another area that really is very important for employers is the social determinants of health piece, I wanted to make sure I brought this up, because a lot of times, employers have focused in on social determinants as a public health agenda. But I think the pandemic has really pulled back the layer on that for employers to the point where they're actually taking a look now at social risk factors, and looking at social determinants that impact their workforce. So it helps them actually begin to ultra-focus, and PCORI researchers, their ability to be able to connect the dots for employers so that they can easily translate that data into action, would be really very important.
- **Anand Parekh:** Great, so many important points there, thank you for bringing those up. Craig. Moving to you, and I think you talked about this already, but from your perspective and your employer's perspective—at a health system, University of Chicago—talk a little bit more about how studies that come out of PCORI, given this new mandate, could be really helpful as you, as part of a health system, try to figure out how to add it as you compare to X versus Y intervention to improve outcomes, which is the one to really invest in. Help us connect the dots a little bit more.

[1:02:43]

- **Craig Umscheid:** Sure. I think, again, first and foremost, enumerating costs of programs that are being compared: that in and of itself, I think, is tremendously helpful for healthcare systems when they're making decisions about investments and return on investments. And you could imagine a scenario where, as part of a toolkit that comes with the results of a PCORI study, perhaps there is a generalizable model there is designed for health care systems to use where you could input if the costs of staff are this, and the improvement and outcomes are that, and our reduction in penalties is this, what is the approximate return on investment?
- So I do think enumerating program costs and maybe creating some generalizable tools that healthcare systems can use to calculate return on investment. That would be a big help. I think the other piece has been shared earlier, but it's worth repeating, just really helping understand patient out of pocket costs. And that doesn't just include dollar signs, or economic costs, but it includes those burdens, which are referenced in the PCORI principles as well, like, what is their return to work after a surgery? So often, as providers, when we're having these discussions with patients about approach A versus approach B, the effectiveness may be similar. But some of the

burdens may be different. And patients ask about those burdens, and as providers, we're often making educated guesses about the impact of the intervention on those burdens.

- So I think having some of that information about burdens, like, you know, time away from work, or time spent in therapy, and being able to share that information with providers and patients, and shared decision making tools that healthcare systems can invest in, would also be tremendously helpful.
- **Anand Parekh:** Great, we are waiting for, audience questions, so, please, send your questions in, and we'll get them to the panelists straight away. While we're waiting, I have 1 or 2 sort of final questions while we wait. The first is: Are there any costs and burdens or economic factors you all think are important to patients and families and caregivers that could be considered an area of analysis given PCORI's new mandate, that we haven't yet talked about today on the webinar. And this is open to anyone.

[1:06:05]

- **Craig Umscheid:** I think one idea that I might put out there, and I'm not sure if this is appropriate per the principles that PCORI has laid out, but understanding how costs differ when a patient is insured by one program versus another. And this goes along some of the comments that people have shared just around digging deeper into inequities. So, what is the cost burden look like if you're covered by, let's say, Medicaid, versus Medicare, versus commercial insurance? How are those costs—out of pocket costs, the costs that patients encounter—different?
- **Anand Parekh:** We have one comment on that question from the audience: "Need to be careful not to confuse cost and price." I don't know if there are any comments on that comment from the panel

[1:07:20]

- **Craig Umscheid:** I agree with that. I agree with that, that's all I'll say. I mean, I think costs, you know, really depend on that perspective. But, from my perspective, on one end, sort of, you know, what are the costs that the patient has bearing? And, you know, with my other hat on, what are the costs that the health care system is bearing, the hospital system is bearing to provide the care? So, I agree very different than a price affixed to a service.
- **Anand Parekh:** Thanks. So one final question for all of you, and we'll continue to look for questions from the audience: What does success look like a couple of years down the road for PCORI, given this new mandate in the reauthorization? How does PCORI take this mandate and, over the next couple of years, fund the research that can really elucidate answers to important questions for patients, for their health care professionals, to do shared decision making, really improve outcomes and reduce preventable healthcare costs, not only for them, but for the health system. What does success look like, when you think about it a couple of years down the road?

[1:08:53]

- **Shawn Martin**
- I think there's success on both sides of this equation. I think certainly empowering patients and their caregivers and their health care professionals and physicians to make more informed decisions that have an element of the economic data associated with it. But I also think there's the other side of this that we're experiencing through the COVID pandemic, which is kind of delayed care, you know, the cost of not acting. And being able to engage patients and caregivers on, you know, the potential economic impact of delaying care further, foregoing care for a period of time or your treatment, whatever the intervention is, I think, is another important

element. I mean, it's a little more sophisticated than “penny wise, pound foolish,” but I do think there also is a cost of inaction that can drive shared decision making.

- **Anand Parekh:** Great. Thanks, Shawn. Margaret?

[1:10:02]

- **Margaret Rehayem:** Yeah, and I actually have my mic open. Anyway, there are two things that I would highlight: one is that the patient, or the end user, is able to navigate the system effectively. I think it's really very important for that to be happening. And there, we talk about cost and quality and value and everything, but at the end of the day, if we aren't really supporting the patient and their ability to get appropriate care at the right time, and that an employer is able to afford that care, then, no, we're not doing the right thing.
- The second thing that I would highlight is the fact that as we're going through this, and the pandemic has brought up so many different challenges in the current healthcare system, but if we could really take a look at how all of these metrics and outcomes interact with one another rather than remaining in our silos, I think we'll all do each other a service by coming together, and PCORI has an opportunity here to really help all of us stakeholders connect the dots. I really look forward to that type of work moving forward.

[1:11:10]

- **Beth Monsrud:** Margaret, I echo that exactly in terms of that interaction. And then I would kind of take it from a plan perspective, is really understanding how all these aspects of burdens and economic impact, the patient's decision making, and how they all interact together. And then, as a health plan, how does that influence, then, the decisions we're making around the programs that we're offering, the way we design our benefits to help facilitate that best outcome and best health? Removing some of, those barriers that are getting in the way of getting needed care and necessary care, at the right place, at the right time, in the right setting.

[1:12:04]

- **Craig Umscheid:** I'd also add on to that, as well. I think healthcare delivery is changing dramatically. I mean, we're seeing it this year, but I think it's been changing and over the next number of years we're going to see dramatic transitions from in-hospital care to home care, virtual care, even in the chronic subacute area, in the mental health area, in the home hospital area.
- So for patients to make these types of decisions and providers, for them to have shared decision making conversations with patients, I think cost transparency is absolutely critical. So, I think that's what that what success might look like in the future, a scenario in which providers and patients have the information they need to make informed decisions, and out of pocket costs are a big part of that. I'll say on the flip side, the same is true for hospital systems. As hospital systems are making more and more investments in these novel approaches to healthcare delivery, really having a sense of what it takes to make those investments and what those costs look like.
- **Anand Parekh:** Great. Randy, did you…

[1:13:35]

- **Randy Burkholder:** Yeah, sure, and I would definitely agree with the last comments. I think in terms of success, as our healthcare system can, and should, and must continue to evolve towards one that places greater and greater demand for accountability, for value demonstration, PCORI success would be playing an increasingly important role in helping to inform that shift, and would increasingly be viewed as a trusted partner for the wide range of stakeholders that we represent, and certainly, that were represented on yesterday's panel, and informing that shift. I think just PCORI continuing to raise the bar for objective, rigorous, patient

centered outcomes research that now can be across a wider range of outcomes, that as they do that, they can help "lift all boats" in the research community and health technology assessment space. And certainly, in clinical decision support was just referenced. I think that's how I would summarize that.

- **Anand Parekh:** Great. Before I let you go, it looks like the questions are coming in now in full force, so we only have a couple of minutes, and then I need to turn it over to Andrea, but I'll just give you a sense of the questions coming in. I think you all have actually touched on most of these, but if there are any additional comments you all want to make.
- One question relates to health disparities: "Should PCORI emphasize that cost impacts be analyzed for health disparities?" The second question is: "Is total cost to patient and system more relevant compared with cost to patient only?" And I think today we've talked about both the out of pocket costs and total cost of care being important. Another question: "I appreciate the focus on indirect costs and costs to patients, but those outcomes may be viewed as secondary or outside the scope of health economists and value assessments. How do the panelists think we might make those types of cost factors more central?" And then a final question: "What's the impact of coverage versus no coverage on certain procedures, drugs, and treatments?"
- So lots of questions spanning sort of health disparities, to how you do some of these studies and, and what we're focused on. Any of those questions that folks just want to further elaborate on? I think we've touched on some of these topics, but I want to give all of you an opportunity to weigh in, and also thank the audience for providing these comments and questions.

[1:16:26]

- **Beth Monsrud:** Now, in terms of the disparities question, I do think that economic burdens and potential barriers from a social determinants of health, and other implications are an important factor about how members engage in the healthcare system in their decisions. And so I do think in terms of understanding some of those root causes and drivers and testing out impacts as to how those decisions are made in varying settings and sets of economic conditions are an important part of understanding how we can make changes to reduce barriers to access to care. I do think it's an important aspect of the research that needs further study and should be part of PCORI's view and incorporated in the data.

[1:17:30]

- **Craig Umscheid:** I wanted to take a bite on that, too, Anand. Similar to my comment earlier, and a couple others' comments earlier, value is clearly, the effectiveness over the cost. And, you know, oftentimes we see differences in effectiveness across different socio-demographics, whether that's race, gender, language, payer. If we also have differences in costs and burdens, the value equation is very different across those populations. And I think it's important to identify those. I wholeheartedly agree with the individual who asked that question, I do think it's important to look at cost effectiveness across these different socio-demographics.
- **Anand Parekh:** Great. Any final thoughts or comments on the audience questions?
- OK, well, I think they're important comments, and certainly are valuable to PCORI. I want to thank all five of you for being just super panelists. Thank you for your leadership and the work that you do. I know your perspectives are important to PCORI and the community. And so, thank you, and with that, I'll turn it over back to Andrew and PCORI staff.

Closing Remarks

[1:18:59-1:20:56]

- **Andrew Hu:** Great, Thank you, and on, and, and for all the panelists for such a great and informative discussion, this is very helpful for us here at PCORI, as we look through the next few months as we collect public comment, public input, consolidate the input that we heard today, and digest the input that we heard today.
- The next step, as I noted, is, is for us to take that feedback and turned into a revised set of principles and guidance for our applicants. So, appreciate everyone providing the detail and getting into the weeds a little bit. It really helps us as we're trying to work through our processes here internally. As Anand noted, we did receive a number of questions that we unfortunately couldn't get to during the discussion. But rest assured, we will incorporate them into our public comment process, and we'll do our best to address them.
- And, also, I noticed today, this discussion was a second in a series of webinars that we had on this topic: yesterday, we held a similar discussion with caregivers, patients, consumers, and the disability community. So, that webinar will be archived and will be available on our website. So if you were unable to make it, please feel free to go look at that video at a later date, as well as this one. And the feedback that we receive from both the webinars and the public comment will be incorporated into PCORI for broader process.
- So, before we close, just the reminder on a few key dates. The public comment period on those principles does close on Friday, November 13th. So if you do have any, please do get them in before then. PCORI will be aiming to update and revise the principles for final approval by our board, and either February or March of next year, and guidance for our applicants will be finalized before the spring 2021 funding cycle. And lastly, I know I speak for Dr. Cook and the rest of the PCORI team in thanking Anand and the entire panel and the audience for taking the time to join us today. We appreciate everyone's engagement and look forward to working with you all as you advance and support patient centered outcomes research. So, thank you everybody, and have a good rest of your afternoon.