THE BROOKINGS INSTITUTION

USING COMPARATIVE EFFECTIVENESS RESEARCH TO IMPROVE THE HEALTH OF PRIORITY POPULATIONS

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Welcome and Introductory Remarks:

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Keynote Address:

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PANEL 1: SETTING RESEARCH PRIORITIES THAT IMPROVE CARE FOR VULNERABLE POPULATIONS

Moderator:

MARK MCCLELLAN
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Panelists:

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RICHARD HODES
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P R O C E E D I N G S

MS. CLANCY: (in progress) -- from the famous pathologist Rudolph Virchow, who once said that all diseases have two causes: one pathological and the other political.

And since we are now into social networking and all this kind of stuff, my Tweet for my comments would be, “Health care reform game change; chronic illness, disparities now.” And that’s where I think CER can make a huge difference.

As Mark indicated this is something that is very, very important to our improving quality and reducing disparities. So, it has been something that we have taken very seriously in our opportunities to invest in comparative effectiveness research and also, frankly, as a way to think about our investments in the use of health IT to improve quality and reduce disparities.

Now, every year we get to report to the Congress on how we’re doing in terms of disparities in health care. Let me just say there’s no shortage of opportunities for improvement. That would be the most positive thing I could say. We report both on quality and access. So for blacks, Asians, and Hispanics, at least two-thirds of measures of quality are not improving. That is to say the gap stayed the same or actually got larger.

For blacks, only about 20 percent of measures of disparities and quality of care improved; not eliminated, but improved. That is to say the gap decreased. For poor people, disparities are improving for almost half of the quality measures, which is good news. This is the first year we’ve seen this. We started reporting in 2003. And for Asians and American Indians and Alaskan Natives, approximately one-third of disparities in quality improved. I could go on and on. For those of you who love data it’s all on our website at ahrq.gov.

And what we know from a recent study by the Kaiser Family Foundation that I thought was particularly breathtaking, focusing on women's health care, is that at the state level quality and disparities are related but very distinct concepts. So, I've been thinking about it in very much similar ways to the way that Mark framed it for us, two sides of the same coin. If you want to actually have a big impact on quality you go where the opportunity is the largest and so forth. But it's very, very interesting because in there, the Kaiser report, high quality does not guarantee low disparities and vice versa.

So, for example, Massachusetts was among the best states involving the share of women who did not get a mammogram; about 16 percent. But the percentage of women of color without a dental checkup was about 80 percent higher than that of white women. In Oklahoma and I think West Virginia, white women and women of color both experience significant problems with access to care and in terms of quality of care, but the disparities were almost nonexistent. This is not the Everest of our ambitions in terms of reducing and ultimately eliminating disparities. But the overall point is that disparities exist in every state on almost all measures.

So, all of you know this. I know many people in this room and I'm thrilled to see you. How do we create a framework for comparative effectiveness research that captures, analyzes, and actually engages clinicians and their patients in these populations in the use of this research? Well, as Mark said -- and I like those slides, by the way. I might be borrowing those. Ours are much more boring looking. The American Recovery and Reinvestment Act, or ARRA, allocated $1.1 billion for comparative effectiveness research. In fact, at the Brookings conference last year, I do recall, this is just when CER was starting to get a little sensitive. And I remember that Senator Baucus proposed maybe we should just call it "Fred." He didn't care, he could see that the work was important, but anything that we could do to kind of diffuse some of the concerns. But the focus on priority populations across the $1.1 billion has been very strong and consistent, and as I'm sure my colleague Richard Hodes from NIH would agree.

In fact, we estimate that about half of the $400 million allocated to the Office of the Secretary was invested in either specific research demonstrations or data infrastructure investments that specifically address the needs of priority populations. I know some of you are waiting to hear more about this. You'll be hearing lots and lots of announcements of initiatives funded over the summer, because we worked very, very hard and long to make sure that three pots of money were very tightly coordinated.

Just to give you a flavor for what some of this looks like at AHRQ, we've invested in research on delivery systems because one thing that emerged this past year was broad consensus and agreements that CER is not just about the clinical stuff, but it's also the care delivery intervention, which is a good thing. So, we are investing in research on various aspects of delivery systems and evaluations emphasizing care for priority populations, which includes people who are members of racial and ethnic minorities, but also people with multiple chronic conditions and so forth. We have a specific initiative that you'll hear about in the near future focused on improving care for people with multiple chronic illnesses. So, it's a very broad focus on priority populations.

Now, if you look at chronic illness care, in particular the question is how do we fix this? Now, where we've tended to see more improvements in quality of care, and even some very promising developments in terms of reducing disparities, is in hospitals, right? Now, the good news about hospitals is although they're chaotic, you've got a captive population. You can have campaigns and blast people by e-mail, signs on the wall, and so forth. Once you get outside the hospital walls it gets a lot more challenging in terms of keeping issues important, keeping it in front of people's face, and so forth. But the Internet and other types of information and communications technology can be very, very helpful in that regard. And yet, we see something of a digital divide. Now, these days the digital divide I think about most of the time is with -- between us: people of my age and the digital natives. But if you actually look at some recent results from PEW, only about a third of people over 65 have used the Internet. About 75 percent of whites have used it compared with 59 percent of blacks. There's also significant differences associated with those -- for those who live in rural areas; presumably some broadband issues, as well as annual income.

So, we've got specific initiatives that we're funding this year designed to at least address the consequences of this divide. One is called iADAPT for Innovative Adaptation and Dissemination of Our Comparative Effectiveness Research Projects. And this will go out to about 25 research teams who are going to come up with and test new ways of presenting this research information. Because if there's one thing we know about CER is the ultimate success of this enterprise will not be judged in terms of peer reviewed publications. That's all important, more better research is really important. The clear guiding intent is that it has an impact on improving patient care. So, I really want to salute the three panels that you've put together here today.

Some of the specific actions I think we can take, and you'll hear about in more detail. One is including data sources for evidence-based studies and diverse populations. A second is following through on the Institute of Medicine's recommendations about collecting data at a fairly detailed level on patients, which I think is terrific. And what's really great news, some communities are starting to do this, right? Cincinnati has got 65 hospital leaders and trainers that they're actually making this part of everyday practice in the hospitals, just as one example. So, that's very, very exciting.

CER has also by ARRA, by AHRQ, and by the intent of the legislation as I would read it is very participatory. And the intent is that there's broad input from multiple stakeholders. I can speak for AHRQ and say over the past five years we have found that fantastically useful. Newell is here and he's been one of the people who's helped us, but not because everyone's giving us point-to-point recommendations, but because they're having a dialogue with each other. So, I'm very optimistic and hopeful about other's who will be appointed to the new -- to the Board of the new Patient-Centered Outcomes Research Institute.

And one thing I'm incredibly excited about is at the National Institute -- I love saying that -- for Minority Health and Health Disparities where they've made some long standing investments in community based participatory research, the Office of Minority Health on behalf of the Secretary will be making additional supplemental investments. And I'm told that they are just totally overloaded with fantastic applications. That's all I can say about it or I'd have to shoot you all.

So, challenges moving ahead I think is not just to make this aspirational, but to actually create a framework and operational plan moving forward where we're making sure that we're getting input from and collaborating closely with those who live in the worlds where priority populations are served, that we've got that prospective as part of our daily work at all times. Clearly the opportunities are going to be discussed here this morning in terms of expanding infrastructure and capacity. I'm thrilled that we're making some investments that way this year in community health centers.

AHRQ will shortly announce prospective studies with a very strong focus on those populations traditionally underrepresented in research. We're going to continue to need innovative CER methods. And we've got to get a lot smarter faster about how do we get this information to people in such a way that good information is impossible to avoid. So, I know that these are all big passions of Mark and his colleagues at Brookings, as well as many of my colleagues who will be presenting in the panels.

So, with that, I want to thank you for organizing this today and I know you've got a tight schedule, so I -- everyone's got to go boom. This is working with Mark. Thanks for your attention. (Applause)

MR. McCLELLAN: Carolyn, thank you very much for your remarks to help get us started. We deeply appreciate them. And you can bet that we'll be staying in touch with all of those efforts that are underway in terms of developing a framework and supporting prospective studies with underrepresented populations, overrepresented on improved methods, on effective communication. I know AHRQ is in the process of getting a citizen's forum up and running, too, as yet another way to have effective input. This really is a major priority now for the agencies. So, thank you very much for your leadership and the opportunity to work with you on these efforts.

And in terms of meeting these challenges, let's get right to that. Our first panel is going to be introduced by Richard Hodes, who is the director of the National Institute on Aging and was probably one of the people -- he didn't know it at the time -- that helped me get started on research early in my career through some NIA funding for issues related to comparative effectiveness, actually. Richard has been long involved in taking steps to not only improve the basic science around understanding the illnesses and health of older Americans and their wellbeing in the community, but also practical steps to identify and support effective interventions and their use. So, he's been a long-time leader in issues related to comparative effectiveness and particularly issues related to priority populations including older Americans and people with multiple chronic conditions and people of lower socioeconomic status and racial and ethnic minorities.

Richard, we're very pleased to have you today to introduce our first panel. Thank you.

MR. HODES: Thank you, Mark. As Mark noted, NIA had the privilege of supporting his career and career development until he exhausted, I think, all possible support mechanisms and decided he had to move along much to the benefit of the nation and the world. And I thank Mark for his opening remarks and for the introduction to this panel. The panel members who have come up to join me -- Garth Graham was not able to join us, so Jamila Rashid --

MR. McCLELLAN: Jamila Rashid is going to be here shortly.

MR. HODES: Okay. Lisa Iezzoni, why don't you come up?

MR. McCLELLAN: And Lisa is a professor of medicine at Harvard Medical School and at the Mongan Institute for Health Policy at Massachusetts General Hospital.

MR. HODES: And Nancy Roizen from Rainbow Babies and Children's Hospital. And Mark's request was that we introduce briefly the topics and expand upon them some as noted. These topics were discussed by the group in some teleconferencing and are reflected in revisions in the draft which you have. So I think there's a need to simply summarize them briefly and elaborate a bit, and then we'll give an opportunity for the rest of the panel members to elaborate as well providing most of the opportunity and time, therefore, for discussion.

So, the first and basic recommendation, developing common definitions of priority populations, is, of course, critical. If we're going to address these populations we need to find them. Now, in some cases this seems rather self-evident, the definitions of certain racial, ethnic populations. Those that are defined by age, are rather self-evident. But in other cases we'll see they are not quite so clear, so the spectrum of disabilities and their definitions, and in the cases of chronic diseases and co- or multi-morbidities are going to be particular challenges in identifying populations. They need to be identified so that we can evaluate their current status, the existence of disparities, and then target CER towards them.

The second bullet recommendation, setting research priorities as related to mission of agencies and departments, is clearly important and, again, I'll just elaborate a bit. In some cases the setting of priorities that are targeted at these priority populations will involve, in large part, inclusion to a degree that has not occurred before. That is there may be common interventions, common studies, common analyses that need to include populations which are more or less applicable to priority populations, but where the status and representation of those populations has been less complete in the past and the challenge is to be inclusive.

There will be other cases where because of known or emerging information about disparities among these priority populations, we'll be talking about targeting specific studies and potentially specific initiatives. So, these are two different areas, but both fall under the important categories of accounting for the populations who, as Mark pointed out, can profit -- will profit most by the successful application of CER.

Then in addressing the needs of priority populations it's important to note that the application of research is going to be directed at both patient and population level decisions. This is, I think to the gratification of all of us, a part of the definitions that have prevailed at the level of federal enterprise in CER including under ARRA; that is, it is broad. It's not simply one drug versus another or one device versus another, but it applies to means of analyzing and delivering health care at the population level, at the societal level. So, this is, for many research enterprises, a continuation of the theme that we've already seen, but it will have to continue to include both optimizing of care of the individual and ways in which to deliver the sum or integral of those individual decisions across populations.

Of interest, the notion of value of information analysis, and this is a clear one of importance, but also very challenging. So, some of the metrics that have been applied in the past have been around concepts, termed such things as "burden of illness," so they have related to the importance and intervention as it addresses the problem at hand. This relates to the frequency of the problem, how many people or individuals are affected, by the severity of the condition as it affects quality of life, as well as financial burden, but importantly, in addition to defining the public health needs and priorities, an important parameter as well is that of scientific opportunity we deal with all the time. So, there will be cases in which there will be needs of high priority, but where scientific opportunities are more elusive or less immediate. And it needs to be emphasized this is not an excuse or rationale for not addressing those problems, but it points to the need for relating CER to basic and translational research. So those areas where the opportunities are still more distant for applying CER, this reflects a need to continue efforts at more basic and translational work and to make sure those are tied to CER. They will not all fall under the definition of CER, but CER will be based upon an intensity of basic research as well.

Gap filling, an important concept. We need to understand those areas which are priorities and of the priorities, which are already being addressed and which are not. And I think the recent exercise we had over the past year stimulated by ARRA, provides some excellent examples of the importance and the challenges and shortcomings of our ability to do this most rationally. So, if one finds the IOM report, which did an outstanding job in a remarkably short time of putting together a list of 100 priorities, they were forced, because of the timing, to do this without the luxury of an opportunity to do a careful portfolio analysis. And, in fact, if they had tried to do the portfolio analysis, speaking for NIH, despite our best and ongoing efforts, it would indeed have been quite difficult to be at a high level of confidence, certain of exactly what is happening. So, to be illustrative of the 100 conditions -- 100 topics that were judged to be the highest priority by IOM, when the National Institutes of Health went through and reviewed its current portfolio before the CER initiative, there were 88 of those 100 which were already being addressed to greater or lesser degrees.

The approaches we took, I think, is an example of what can occur in response to omnibus or blanket, broad solicitations for CER applications under ARRA. We were able to address not only some of the 88, which are already actively supported by additions, and when then a few months ago we assessed to see what gaps remained, we found that over the 25, that is the top quartile of the IOM 100, there were three areas of research which were not being addressed, and so we're able to issue now a targeted funding opportunity. Had outstanding responses by investigators in those fields and are now in the process of reviewing those. So, we have to be committed to a better understanding of just what research is ongoing and monitoring that in real time. And as we succeed in some areas, continue there, but also make sure that efforts are directed at those initiatives where we are falling behind in terms of success and moving forward. So, gap filling is going to be important, but it's got to be done in the context of improved informatics and analysis of where we're making best efforts.

So, Mark, I hope within time constraints, I thought we'd stop there and move along to give each of the other members of the panel an opportunity to comment briefly and introduce themselves. Jamila, welcome.

MR. McCLELLAN: Jamila, thank you for being here. Jamila, for those of you who don't know her, is the associate director of the Office of Minority Health at HHS, where she oversees research programs. And because of a conflict with Garth Graham, she graciously agreed to be here on short notice. We really appreciate your joining us.

I'd like to start down at the end of the table and just go across the table to follow on Richard's excellent summary of the recommendations, and I'll start with Lisa for her views.

MS. IEZZONI: Okay. I had the brief about people with disabilities. There's about 54 million Americans currently living with disabilities in the United States. That's about 20 percent of the population. I just want to make the point that disability rates are much higher in some of the vulnerable populations that we're talking about here today. Obviously the elderly, but also racial and ethnic minorities typically have higher rates of disability, especially African Americans and Native Americans have very high rates of disability.

Now, when I used the words "54 million," that's a lot of people. It's obvious that disability is an incredibly diverse concept. It ranges all the way from children with developmental disabilities to elderly people with Alzheimer's disease, to people in middle age with spinal cord injury. In terms of thinking about comparative effective research, the phrase "people with disabilities" really is not an actionable phrase. It really needs to be honed in, focused on, for us to be able to begin to, in a meaningful way, identify where the gaps are for comparative effectiveness research.

Now, another problem for doing research on people with disabilities is that information about disability is typically unavailable in the traditional data sources where people go to look for it for comparative effectiveness research. It may be kind of shocking to hear this, but even medical records may not very extensively document disabilities such as hearing loss, vision loss, if it's not actively being treated by the clinician. The National Center for Health Statistics surveys do collect pretty good information about disability, but the traditional discharge abstracts, the claims that are analyzed by so many investigators to look at service use, do not have any information on disability in them.

People with disabilities can require health care interventions on three levels which require comparative effectiveness research. The first is routine health services that everybody needs such as screening and preventive services, and care for episodic illnesses. The second is care for their underlying disabling condition such as arthritis, which is the leading cause of disability among adults in the United States. The third is care for comorbid conditions that occur, for example, people with disabilities can also get breast cancer and colon cancer.

Comparative effectiveness research studies for the first and third types of interventions have been conducted, you know, studies for breast cancer, studies for routine immunizations, but typically people with disabilities are explicitly excluded from the clinical trials that are used to create the scientific evidence base. Therefore, the research evidence may not, in fact, apply to certain people with disabilities.

It’s important to note that some disabilities affect people fairly little in terms of their anatomic or physiologic functioning. For example, if somebody is born blind or born deaf, they may not react any differently to breast cancer treatments than other people. But for somebody who had poliomyelitis as a young person, they may, in fact, respond very differently to breast cancer treatments. And so for certain subsets of populations with disabilities, it’s important to do specific analyses on them.

Finally, it’s important to know, and I’m going to be a little bit political here, that it is important to do studies on interventions that our health care system currently may not pay for because, in fact, those may be the exact kind of interventions that are going to be most likely to improve the quality of life of people with disabilities. For example, the medical necessity provisions of Medicare and many private health insurers refuse payment for services that are viewed as convenience items or that are not actively improving or restoring function. We don’t have much comparative effectiveness research on physical or occupational therapy that is meant for just simply maintaining function or preventing its physical declines. Wheelchair technologies with capabilities that are deemed not medically necessary need to have further research on them because, in fact, wheelchair technologies like that can significantly improve the quality of life for people with severe mobility problems.

Finally, home modifications could improve the ability of people with disabilities to live independently in their communities and to participate independently in their daily lives, but home modifications also are not often paid for and may not be under the kind of purview of comparative effectiveness research because of that.

And so, that's -- my comments are about done, but that's where my focus is this morning, on disability.

MR. McCLELLAN: Lisa, thank you very much for your comments. And now I'd like to turn to Nancy Roizen.

MS. ROIZEN: Eight to one, the return on investing in the families and the education of young, disadvantaged children is 8-to-1. That is the conclusion of the University of Chicago Nobel Prize-winning economist Dr. James Heckman. In studies investing in the families and the education of young disadvantaged children, the return on the investment of $8 -- was $8 for every $1 of investment. This is in his article in the 2009 *Economics and Human Biology*, probably not very many of us read that, and in the 2006 *Science*. But maybe you read the January 2006 article in the *Wall Street Journal* by Dr. Heckman entitled, "Catch 'Em Young." He wrote, "There are many reasons why investing in disadvantaged young children has a high economic return. Early interventions for disadvantaged children promote schooling, raise the quality of the workforce, enhance the productivity of schools, reduce crime, teenage pregnancy, and welfare dependency. They raise earnings and promote social attachments." Or as he goes on to demonstrate, "The benefit/cost ratio is 8-to-1."

So, why are only 20 of the IOM's selected research priorities for CER related to pediatrics with maybe 25 more related to adults and children? But how many will actually go to children?

Maybe it's already been done. No, pediatric effectiveness research, even research in choosing the best intervention for children identified with a hearing loss at birth, hasn't had much funding, which actually happens to be topic number one on our list. Actually, this is a perfect topic. Thanks to the 1998 work of Christie Yoshinaga-Itano, we know that if we intervened with amplification and education by six months of age, we take advantage of the sensitive period of brain development and these children will have language in the typical range instead of half their age. But we do not know what is the best intervention for each child, so I would give a big vote for number one.

Maybe children are not a priority group or a big enough group. Hardly. Twenty-four percent of Americans are children. Forty-three percent of children represent racial and ethnic minorities where there are huge shocking disparities in health and health care of children as described in the *Pediatrics* article in March 2010, with higher mortality from drowning, leukemia, congenital heart disease. And if you have Down's syndrome, you die 25 years earlier if you're a minority than if you're white. And 17 percent of children have a disability. They also have chronic diseases, so they represent four of the priority groups.

So, will children, where you get your biggest bang for your buck, get their due? Were they well represented on the 23-member IOM committee? I don't think so. Only 1 of the 17 physician members on the committee was a pediatrician. Thus the message that I mean to relay to you is, one, studies lumping adults and children cannot concentrate on the period zero to two years of age when the brain depends on early experiences to develop incredibly important sensory and language pathways or synapses that we then spend the rest of our life pruning away, or the zero to five period when Dr. Heckman has told us that the returns are great. This class, children, need to be considered separately so we can get the data, like what Lisa has said, that can make these disadvantaged kids taxpayers.

Two, there currently exists very limited clinical effectiveness research in children, but we should fix this problem and not perpetuate it. We need to fund clinical effectiveness research and the best and the brightest will come and do the work. And the topics on clinical interventions, like topic number 10, which starts in the prenatal period, have the enormous potential to benefit society.

And three, if we start by making children, who are 24 percent of the population, a truly top priority and give them a disproportionate share, one, we can capture the minority priority group as 43 percent of children are minorities; two, we can capture the disability group as 17 percent of children have a disability; and three, we can change their trajectory so that as adults they can live better, have better health and education, have better occupational outcomes, and make our society more globally competitive with the Heckman 8-to-1 return on investment and help these kids be taxpayers.

MR. McCLELLAN: Thank you very much, Nancy. Clearly, you know a lot about the issue and feel very passionately about it.

And I'd like to turn to our next panelist who also has a long-time commitment to leadership on issues related to comparative effectiveness for vulnerable populations, and that's Jamila Rashid. And again, appreciate your stepping in on short notice, Jamila.

MS. RASHID: Good morning. Yes, very short notice as of yesterday evening. So, please bear with me if I ramble a bit, but I want to just say that you're going to hear a common theme here across my two previous speakers and myself, and there is a link as well between racial and ethnic disparities and disabilities as well as with children.

So, I just want to highlight a few things. One, I think it's not a mystery to most of you if not all of you that the racial and ethnic minority population is growing steadily and by 2050, it could likely be as high as 50 percent. And that sends a message to all of us that as we develop these initiatives and programs that we have to more and more take into consideration the special needs of these populations so that we can better assure that what we're doing will have a greater impact, because it impacts on the lives of all of us.

I think it's no mystery to many that racial and ethnic minorities are not only impacted by the fact that they are minority groups, but they also are impacted by many social determinants of health, many things that keep them from having the same equity in health that other populations have: poverty, low education, poor housing, environmental constraints, neighborhoods that are not adequately -- do not have the adequate infrastructure to meet their health needs, and so on. And these factors play a role in how and whether CER will actually work in these populations, and we have to take that into consideration.

In the Office of Minority Health we are working on CER. And we have looked at the IOM recommendations and we've identified those that we feel that we can use to make a difference in the lives and health of the populations that we serve. However, there are others that as we begin to work on this we're going to have to go back and try and see how do we address some of the other concerns and needs.

One of the issues that was raised earlier about persons with disabilities is also for racial and ethnic minorities and that is their lack of adequate inclusion in clinical trials and in research studies. And this was pointed out by the National Medical Association in one of the first -- I believe the first listening session that the council held, and that cannot go unnoticed or lost in the work that we do.

Another point that I want to make is that the need to make sure that we engage the populations because of their special needs, and they differ: American Indians are different from Hispanic-Latino and different from African Americans. And so what might be needed -- and that population could be different or slightly different from what is needed for African Americans. So, within the Office of Minority Health, we're using a full-scale engagement approach to how we do CER. We're also using a professional development approach where you take experts, those who are experts in CER, and involve them with established academic researchers and community researchers to work together in a collaborative way as opposed to having someone do something to these populations. We want the populations to be a part of that and we think that research -- that should and could possibly be part of CER research -- actually studying how best to get communities and get populations to receive, accept, and use CER rather than just disseminate it and put it out there, but make it usable.

Another key point -- and I don't know if I'm running out of time or not -- I can see far without my glasses, but I can't see far with them.

MR. McCLELLAN: You're okay.

MS. RASHID: Okay -- is that we want to make sure that, as I said, that we provide education. When we start our activities we will spend time doing training, doing education, helping communities to understand what is CER and how will it help them. And then we want them to help us figure out what is the best way to get that information to you. What are the reading levels that you need that information at? How can we use social networking to help you get that information? I think this, in itself, is a body of research that we could better understand what do we know about how often when people receive materials in the mail or on the Internet. It actually impacts on whether they go to the doctor and it results in an acceptance and a change in their behavior around CER-related activities.

You're looking at me now, so I think that's my clue, right?

MR. McCLELLAN: It's okay. If you have anything else important to say, Jamila, please go ahead. I certainly don't want to make you nervous up here with all the beeps and the looks.

MS. RASHID: I'll just wrap up by saying that we know that there are certain health conditions, there are leading causes of health -- we're putting our focus on those within the Office of Minority Health. And we hope that we will be able to show that by engaging the populations that we want to accept CER in what we do, that that in itself will be research that could be used to inform CER going forward.

MR. McCLELLAN: Jamila, thank you very much and, again, thanks for getting through the beeps and things to be able to participate here this morning.

I'd like to start off the discussion with a couple of follow-up questions on all of your comments and on fleshing out these draft recommendations, and I'd like to start with just the definitions here. The first recommendation in this set focuses on a common definition of priority populations and the implementation of that definition. And you heard from Carolyn that there are some efforts underway now to actually collect data as part of research studies on race, ethnicity, other important characteristics relating to identifying priority populations reliably, but there are a couple of challenges here.

One is that if you look across the different efforts to promote more effective development of evidence for these populations, there are some differences, maybe some would argue they're not huge differences, in what the definitions are. So, the economic stimulus legislation, the recent health care reform legislation, the IOM and OMB have had somewhat different definitions of vulnerable populations. And I'd like to start by asking if, first, any of you all have any comments about whether these issues are settled enough. And for practical purposes, at least, we can move forward clearly on conducting further studies in a way that will be comparable, and that rests on some common foundation of definitions. Or is there more work to do there?

And then second, any promising next steps for actually making sure that these populations can be reliably identified and data on them can be collected in practice, since that seems like a kind of prerequisite for being able to learn something about the affects of the treatments.

Any thoughts? You want to start, Lisa?

MS. IEZZONI: Yeah, sure. It's a huge issue for people with disabilities. First of all, disability is not a static concept. You know, people's functional status can change over time. You know, gender usually doesn't change and, you know, people's self-identified race and ethnicity can change in how people identify it. You know, the Census Bureau showed that when they looked at different ways of collecting information on race and ethnicity over time.

But I think one of the issues for disability is that the typical ways of kind of making data into bits, the International Classification of Diseases, 9th revision, Clinical Modification, ICD-9-CM, which is the classic scheme for classifying diagnoses just simply does not apply to disability. And the World Health Organization, which is the über organization that oversees ICD, has a functional status classification system. It's called the International Classification of Functioning Disability and Health. But for some reason, the United States just has not picked up on it and there certainly are problems with it. I can't say that it's a perfect classification scheme, but although ICD-9-CM codes appear in every discharge abstract, in every claim that's submitted by providers for reimbursement, there are no ICF codes, no functional status codes.

You also have the issue that some people may not want to self-identify their disabilities because they may be afraid of discrimination, they may be afraid of revealing things that they don't want other people to know about them, or, in fact, they may not feel themselves as having a disability even though they may have a condition such as deafness that, you know, may be viewed as a cultural linguistic condition rather than a disability whereas the rest of the world might.

And so I think for disability, maybe we should just say people with disabilities and leave it undefined so it's included as a category. But for operationalizing it and then moving on to make actual decisions about what exact type of research needs to be done, we need to think both about collecting information and being more specific about the conditions that are included.

MS. ROIZEN: When I think about how are we going to define things, I have a couple of comments. One comment is, it wasn't clear to me where poverty fit in this equation. It seems like it should be in the equation someplace.

Secondly, in relation to disabilities, I agree with everything that Lisa has said. I think you have to break things down into specific disabilities and you have to look at what you're doing specifically about high-impact, low-frequency disabilities such as deafness and high frequency, we'll call it low-impact disabilities such as ADHD. And you need to -- you really need to break them down.

I think in relation to children that the issues are very different when you're a teenager, And the issue is -- what we're trying to look at is suicide or depression; or when you're zero to three and where you, as I have already pointed out, have the opportunity to get much more of an impact for your input, but that there are different ages. And I think the IOM list does identify different ages and it's important in children.

MS. RASHID: Yes. In terms of definition, we could look at groups of categories, you know, by race and ethnicity, by age, gender, geography. There are many different categories that could put someone in a priority grouping for CER, and that would be one way of doing it. Another thought that comes to mind for me is looking at criteria such as need and opportunity. What is the need? Is there a need there for CER to improve health and health care for a particular group of people, and what are those needs that exist? And we may want to look at that. And then is there an opportunity? An opportunity to intervene? An opportunity to identify strategies and ways of disseminating CER, and those sorts of criteria that could also come into play? But I do think there is some value in not necessarily have it drive decisions, but at least it should be examined and explored and taken into consideration.

MR. HODES: I think consistent with what everyone has said there's certainly an importance to identifying the characteristics of individuals who we currently are assigning to priority populations. But I think even more important than identifying those populations with rubrics or titles or labels now, and using those then to define research in the future, is the establishment of a database, of an evidence base, that will allow what is likely to be a discovery of relevant priority populations and sub populations as we evolve. So, I think the real challenge, Mark, is in the database, be it in health care in general or in research, to identify a common database that will allow an assortment and assignment to groups and populations as research dictates and as needs dictate.

MR. McCLELLAN: And do you see a mechanism for doing that now that the comparative effectiveness research is really starting to take off and there is so much attention to getting the infrastructure right?

MR. HODES: Well, some CER is going to be carried out in randomized clinical trials where there's a great deal of flexibility in identifying what the minimum dataset will be. But a large component, as you alluded to, is going to be -- and as Carolyn mentioned as well -- is going to occur in observational studies, which are going to take huge advantage of health IT and health care reform. So, I think it's part of the -- in those large rubrics is really the place where we need to try to exert influence in establishing a common clinical database that will be accessible for this kind of research.

In addition to identifying populations where it's clear there are special needs, we are going to uncover, undoubtedly, in research cases in which variables of age or disability or race or ethnicity were not suspected to have any relevance, and they will, and we're going to have to redefine groups there.

But if I could just identify one example that occurred over the past few years, there was a study carried out, supported at NIH, to look at comparative effectiveness for interventions to prevent diabetes in those who are at high risk for diabetes; high risk based on clinical diagnosis, short though of chemical definition of diabetes. The groups were assigned into three treatments: one was placebo; the other was an oral hypoglycemic, a drug; and the third was behavioral intervention.

At the time the study was put together there was a suggestion that because individuals at older age groups were likely to have more co-morbidities, might be harder to recruit, that there wouldn't be any effort to oversample. In fact, they might be restricted to younger adults without concomitant diseases, and that's when there was some advantage to NIH and its heterogeneity. So, the Aging Institute, in collaboration with NIDDK, worked to help assure there was recruitment over a wider age range, including older adults, and the results were not anticipated by any of us.

So, in young adults, oral hypoglycemic and the behavioral intervention, which was exercise and diet, were equally effective in reducing by approximately half the risk of diabetes. This was one of those trials that was stopped early, not because of adverse effects, but because it was deemed to be so positive that it was unethical to continue people in the placebo group.

In contrast, in the age group 65 and older, the drug, the oral hypoglycemic which was effective in young adults, had absolutely no effect. And an even more impressive and, fortunately, positive contrast, the behavioral intervention was more effective in older adults than anywhere else, producing a 71 percent reduction. And this group, by age, was at highest risk for diabetes. I don't think that anyone predicted the result or could have, but it's a point to emphasize what we've heard from all the panelists, the need for people being able to identify groups -- with age, it's relatively easy; with other groups it's going to be more difficult -- and then inclusion. If we don't include individuals in these various heterogeneous groups, we'll never determine whether one or another intervention is better for a particular group or subgroup.

The extreme of this (inaudible), I'll quickly add, is personalized medicine, when we take full cognizance of the importance subgroups to be careful not to regard them as homogeneous within a group and eventually be able to look at individualization within these priority populations.

MR. McCLELLAN: Thanks, Richard. One more question and then I want all of you to be ready with your questions and comments. We're coming to you next.

This panel has been focusing on priority setting for priority populations and in the comments that you all have already made, in the report, there are a number of ideas for ways to approach the priority setting process that assures that the most important opportunities are directed. That said, I wanted to push you all a little bit further on ways in which the -- specific ways in which the priority setting process could be improved and what -- perhaps what some of those priorities might be. So, as the Patient-Centered Research Institute gets up and running, any one particular piece of advice you'd want to give them about priority setting based on the experiences we've had so far.

So, for example, Jamila talked about the possibility of having a more community-based process for defining priorities with the hope that that would not only make the priorities more relevant to the populations that we're focusing on here, but also help with communication and acceptance and that's something that AHRQ is starting to do now.

And another issue, I can tell you, in the IOM process, there was some discussion about just what was the scope of this health care-related comparative effectiveness research. Was it about traditional medical interventions? Or should it include, as Nancy rightly emphasized, should it include the fact that many of the interventions that make the biggest difference for especially younger children are not what you might think of as traditional health care, or at least not simple traditional health care? So, a quick idea from each of you on making sure that the priority setting process going forward reflects the kinds of goals that this panel has stated.

MS. IEZZONI: I'm hoping you'll ask me last, Mark, because it's just so hard around disability because the population is so diverse, and Nancy made the compelling case about children who are born deaf, to intervene before six months of life. I mean, how could you argue with that not being a priority and trying to have a priority about how to make that happen?

But then at the other end, I also made the point that we need to emphasize research that might go beyond the boundaries of traditional health care to look at ways to improve quality of life for people with disabilities, looking at, for example, smart homes and technologies that allow people to live more independently in their homes. And so it's just, again, very, very hard for me with the brief of people with disabilities to get too specific because the population is just so diverse, and I'm sorry to wimp out on you that way.

MS. ROIZEN: I think that -- I mean, even though as you clearly have heard, I argue very strongly for children to be a top priority, if not a disproportionate priority. I also argue for balance. I think there needs to be -- everybody needs a chance. And my sort of vision of how you set your priorities is that you have, you know, the high impact and you have low impact and you have high frequency and you have low frequency and you have safety, and then you turn it the other way and you have safety and you have possible benefit. I mean, what possible benefit are we expecting or hoping for? And that we look at it in a multidimensional way and that people have to, when they're -- everything doesn't apply to every situation, but that when people are looking at priorities and applying for these grants that they have to at least address these different aspects, these different dimensions, and it should be sort of multidimensional.

MR. McCLELLAN: And in terms of scope, it sounds like pretty broad as long as there's a significant -- I mean, you're really focusing on the impact on health, right?

MS. IEZZONI: Well, I think quality of life, too. I mean, wouldn't you?

MS. ROIZEN: Quality of life and participation. That was the word that you have used. You know, do you have a job? Are you participating in your community? We have to do better in being competitive as a nation.

MR. McCLELLAN: And things like early education intervention's clearly falling within the scope.

MS. ROIZEN: Yes, absolutely. I mean, you know, the opportunities are just -- the earlier opportunities, the data is so -- the amount of money that you have to put in for later opportunities is a lot more for an effect than earlier opportunities, if you get it right. I mean, you might not -- if you get it right.

MS. RASHID: I worked in the National Immunization Program for about five years at CDC before doing some other things there, and we had some terms we used a lot, one being "low-hanging fruit," and trying to focus on pockets of need. And I think we have some low-hanging fruit within the list of CER priorities, IOM priorities, and then there's some that, of course, may not even be on the list. And so I think there is some value in looking at what are some of those interventions and less important ones on the list that should maybe be brought up to the top because they're opportunities for us to maybe show whether they're effective or not effective.

A lot of resources, for example, by some states and agencies are being used on community health workers, on patient navigators and those sorts of things, and so those are some areas that perhaps we should look at those types of interventions and see whether we should put some time and energy into really -- or raise them up to really tell once and for all, is there value in those or are there only value in them for certain types of populations? That would be really valuable to this whole country to know that with these types of groups, yes, community health workers are very important and very effective, but with these others, use something else. Then we could better tailor the use of our resources if we understand that. Too often we kind of put that on the back burner because it's not sexy or whatever, it's not as science-based. But they may really -- it may be some real value in understanding once and for all what do we -- what can we get from that, if anything, and who should that be used with.

MR. McCLELLAN: Any further comments?

MR. HODES: I think we've heard excellent comments and I don't want to take any time needlessly repeating them. Just note that another challenge we're going to have is to do a better job of being aware in real time of what research is ongoing. Duplication in some areas can be useful, but I think there are a number of areas where research has been quite inefficient because of absence of realization of what is ongoing, what is being funded. Talk about low-hanging fruit, this is an easy thing we ought to do. We ought to make sure there's a centralized awareness and database of ongoing research studies.

And just know, we've had a fair bit of experience these past years in trying to use priority setting. Can't argue with the fact that public health needs and scientific opportunity are the two basic contributants, but in terms of public health needs, we're going to undoubtedly be facing some difficult decisions which involve rather subjective evaluations. And whether it was qualees or other measures which have been discussed over the years, there have been controversial assumptions that have weighed, for example, the relative value of life and quality of life and years of life at different stages in life. I think we would all like to avoid competition over which age, which condition, is somehow more of higher value than other, but I think there's going to be unavoidable challenges that we face.

MR. McCLELLAN: Good. Excellent comments. Let me ask you all to add to this discussion now. If anyone has a question or comment, please raise your hand. Wait for a microphone, which will be coming to you in the back first, here second. And again, be sure to identify who you are when you ask your question. Thanks.

MR. MILLMAN: I'm Mike Millman from the Health Resources and Services Administration, and I just wanted to mention that we were successful in getting some of the Secretary's $400 million, and I wanted to mention three projects that are incredibly relevant to the topic this morning, two projects and these are projects that are now solicitations out on the street soon to close. Two are in the area of pediatrics: one is focusing on pediatric emergency services and the other is focusing on pediatric office-based practice research systems. And in all of the cases I'm going to mention, the money we got was for infrastructure building, and a big sort of focus of that is going to be how do we use the movement to electronic health records and information exchange to be able to use for comparative effectiveness research.

The third project is I think very unique, and that's -- we're calling Community Health Applied Research Network, and we're going to be funding four research center nodes. These are community health center networks and other community providers that are going to link up with academic affiliates and one of those is going to be in the area of HIV/AIDs.

So, with the disability here -- the way we argued for this was kind of the Willie Sutton approach, you rob banks because that's where the money is. And we argued if you want to address underserved, vulnerable populations, whether you cut it in terms of economics or racial and ethnic minorities, you go to HRSA programs. And so I think we're in the beginnings of this, of trying to take advantage of some of the infrastructure building. We've already started the movement to electronic health records and an effort to try to figure out how to do comparative effectiveness research, which I think is the other major issue. As we were constantly reminded during the sort of priority setting process, it's not just about doing research or health services research, but figuring how to do comparative effectiveness research which has a lot of methodological and data issues associated with it.

MR. McCLELLAN: Thanks for the comment. Any comments? Definitely appreciate the work on priority setting.

We had a couple up here, Jonathan and one over there.

MS. CARLIN: Hi, I'm Roberta Carlin from the American Association on Health and Disability. And the presentation was excellent, thank you.

I just have one comment that I think reflects the comments of the presenters about setting definitions for priority populations and in the draft document, combining disabilities and multiple chronic conditions, I would recommend that those be separated. They're really very different and as Lisa said and was followed by the other speakers, just defining disability for purpose of CER in research is complicated enough as it is and then to somehow attempt to integrate chronic health conditions I think is doing a disservice to both priority population areas.

So, thank you.

MS. McCLELLAN: Comments? Lisa? Lisa agrees.

MS. IEZZONI: Yeah, I agree with that.

MS. McCLELLAN: And it does get back to a point that Jamila made which is, look, if you take these priority populations together, we're really talking about something approaching a majority of the population. I mean, this is a big part of the overall population, the health opportunities. This is where the money is in terms of opportunities to improve health and have an impact. And that really does suggest a different -- a higher degree of attention to both the definitions and applying the definitions, collecting the data and research, as Richard said. And, you know, I think that’s going to be a pretty big challenge given the scope of the work here.

Do you all think we’re up for it?

MS. RASHID: Yes.

MS. IEZZONI: We have to be.

MR. McCLELLAN: All right. I know Jonathan had a comment here.

MR. HARE: Jonathan Hare from Resilient Network Systems. First of all, great presentation. I have a question.

I’ve looked at a lot of the plan designs and study designs for comparative effectiveness research and it seems like the first step is de-identify the data so we can get the rights to use it. And when you do that, you basically lose the ability to correlate that data with other datasets, things like behavioral background, socioeconomic status, disability status, just about all sorts of stuff. And I look at the definition, you know, what are the definition of the priority populations? How would you query the database and sort of filter out the priority populations if you’ve eliminated any ability to sort of cross index with other sources? How do you eliminate confounding errors if you can’t capture -- you have no ability to capture the data from these other sources?

And on a related topic, how do you then use this intelligence and make it actionable by linking it into care deliver? I think if you were to add up all the money spent on comparative effectiveness research, it’s got to be a fraction of 1 percent of overall health care delivery. And if you’re not able to integrate it back, once you’ve de-identified the data, you do some analytics, it’s basically impossible to reach back out to the caregivers and the patient, add any value, and get sort of a feedback loop.

So, the question is for Richard and Mark, since Mark knows everything --

MR. McCLELLAN: I’m just the moderator.

MR. HARE: Has there been any progress in sort of a systematic way for capturing data from diverse sources, sort of not irreversibly de-identifying it, using it, and then linking it back into care delivery environments?

MR. McCLELLAN: Well, as the moderator, before putting the rest of the panel a little bit on the spot about this, just a reminder: I mean, those are good questions and we're going to come back to them in our next panel on infrastructure for conducting the research and then for our third panel on using the evidence that's developed, but these are issues that relate to priority setting, too, and they're important challenges and priorities will hopefully find a way to address them.

Any comments?

MS. RASHID: I have an easy comment. We'll send our folks that are going to do that to the next panel and they'll deal with it there, because it is a bit of a challenge.

MR. McCLELLAN: Lisa?

MS. IEZZONI: Yeah, I do have a comment and I'm not sure I'm going to be able to say this crisply, so I apologize, Mark, in advance.

I personally believe that the view of the person with a disability about their functional impairment has inherent validity, but a lot of physicians say, oh, no, that person's too subjective, they can objectively evaluate their disability, and so they insist on having a physician come in and evaluate the functional status of somebody. But, in fact, physicians often see patients at their worst. You know, they're sick, they come into the office because they're feeling poorly. They don't see them when they're out in the community, when they're living their daily life, and so they really do not have a good perception of exactly what the functional status of that person is.

And so I think whatever data systems are created need to be able to include the perspectives of the person with disability about their own functioning. And, in fact, the small body of research that has looked at this has shown a lot of discordance between physicians and patients in terms of self-rating the patients of their disability and the physician's ratings. And again, as I said, I think patients have an inherent validity there. And so whatever data systems are designed need to maybe include both perspectives, but I think scientists need to increasingly respect that the view of the person with the disability needs to be, at the end of the day, the view that dominates.

MR. McCLELLAN: And indeed, with this greater emphasis on personalization, as Richard was saying, and more reliance on individuals for providing the data, seems like something that could be reflected in the priority setting.

MS. IEZZONI: Yeah, but it's going to require a mindset among our profession, change. Yeah.

MR. McCLELLAN: Richard?

MR. HODES: All good comments. In terms of subject reports, you know, I think there is some progress being made defining the right query instruments that are amenable to data-basing is important and there is some progress. But the basic challenge, I think, is always going to be there: trying to protect the rights to privacy with the value of having extensive information that is ultimately potentially identifiable as a fodder for research. And there would be certainly -- and Mark probably does know better than anybody else from experience here -- but there've been a number of approaches taken that will continue to be needed.

There's the use of enclaves and confidentiality agreement that under highly regulated circumstances allow limited access to data which is potentially identifiable, but with very stringent restriction to access to those data.

There is the format of informed consent, so I think -- you know, Joel will be talking about VA. There are opportunities there for large populations who may choose to participate with rather global consent. Informed consent is a challenge here, but individuals who are willing to ease, if you will, constraints on personal identifiers in the interest of research, but there’s never going to be a way, I think, to avoid the importance of respecting confidentiality in individuals and having the very most informed consent when that is being compromised in any way.

MR. McCLELLAN: And we are going to come back to these important methodologic issues in the later panels and the further work on this effort.

We’re just about out of time. I know there are some more questions. I know there’s one here and we’ll see if we can get through that, and may not get to all of them here, but we’ll have more time for discussion in the later panels.

MS. TURK: Thank you. I’m Peggy Turk from SUNY Upstate Medical University in Syracuse, and I’d like to thank the panel for the presentation and starting us off, I think, very nicely.

I have a question in regard to that dirty word called “cost,” and that is that people have talked around it, have mentioned it briefly, and yet I am very much aware of issues of cost because of my practice, I’m a physician in physical medicine and rehabilitation in pediatrics. And what we see is that cost of care is really one of those limiting factors. And so it seems to me that that might make your list, Nancy, of what is the high and the low impact, and yet I know we don’t talk very much about cost.

Lisa, I think you also mentioned some cost issues as well. But I wonder if the panel might make some comments recognizing of course we’re looking for effectiveness of interventions, but would cost have any impact as well?

MR. McCLELLAN: I think cost is an important issue. What do you all think?

MS. IEZZONI: In disability, Peggy, I agree, cost is an important issue, but it's also cost beyond the patient. It's cost to the families, to the caregivers, to the community. And so if we're going to go around cost for people with disabilities that we need to -- it's like putting the pebble in the pond, we need to look at the ripple effects.

MS. ROIZEN: Once again, I think that it's -- as you say, it's the elephant in the room, so to speak. And I think your comment to low-hanging fruit was a comment to cost. Let's get the easy, quick, cheap things done and I think it has to be in that multidimensional equation. And then we have to consider what is this going to mean for a whole life or a whole society if Suzie gets her cochlear implant and she can be sort of mainstreamed forever, is it worth the cost?

MR. McCLELLAN: It is the elephant in the room, and if it's the elephant in the room, it's better off acknowledging it in the process.

MS. RASHID: I know you're short on time, but I just want to echo Lisa's comments. There are other non-monetary costs that we have to factor in.

MR. McCLELLAN: Very important. And we are short on time. I know there's some more questions. We're going to try to hold them for the upcoming discussion, but right now I'd like to thank our first panel for doing a really good job of getting us started.