

**Patient Engagement in the Design and Conduct of Medical Device Clinical Studies - Final Guidance  
March 22, 2022**

**Moderator: CDR Kimberly Piermatteo**

**CDR Kimberly Piermatteo:** Hello, and welcome to today's CDRH webinar. Thank you for joining us today. This is Commander Kimberly Piermatteo of the United States Public Health Service, and I serve as the Education Program Administrator in the Division of Industry and Consumer Education in CDRH's Office of Communication and Education. And I'll be your moderator for today's program.

Our topic today is on the final guidance titled Patient Engagement In the Design and Conduct of Medical Device Clinical Studies. As you'll learn more today, FDA acknowledges that patient engagement may be beneficial across the total product life cycle, and this guidance focuses on the applications of patient engagement in the design and conduct of medical device clinical studies.

We're holding this webinar to provide you with an opportunity to learn more and to answer any questions you may have about this final guidance.

It's my pleasure now to introduce you to our presenter for today's program, Tracy Gray, Patient Engagement Lead within the Patient Science and Engagement Program in CDRH's Office of Strategic Partnerships and Technology Innovation, or OST. We'll begin with a presentation by Tracy and then field questions about this topic.

Thank you all again for joining us today. Now let's hear from Tracy.

**Tracy Gray:** I'm Tracy Gray, the Patient Engagement Lead in the Center for Devices and Radiological Health, and I'll be giving an overview of the guidance.

This slide includes links to the final guidance and the docket.

The objectives of this guidance webinar are to describe the background, development, and contents of the patient engagement guidance, discuss the meaning of patient engagement and how patients as advisors can help improve clinical study design and conduct, review examples of opportunities to engage patients as advisors, and identify helpful resources when developing patient engagement approaches in clinical studies.

Now let's go over some background information that's relevant to this guidance.

Patients are experts in their conditions and offer valuable information about living with the condition and its treatments. These perspectives can significantly impact the development, evaluation, and monitoring of medical devices. Benefits of hearing from patients could be experienced at all stages of the medical device product lifecycle, including informing CDRH's thinking on how current issues impact a patient community, such as COVID-19, designing medical devices with insights on unmet needs or usability issues, planning and conducting medical device clinical investigations, which is the focus of this guidance, helping to identify or refine emerging safety signals, more effectively communicating with affected patients about recalls or other safety messages, and identifying specific populations' perspectives on benefit risk for a given treatment.

The development of a framework for this guidance is an important outcome of the Patient Engagement Advisory Committee, also known as PEAC. The PEAC is the first and only advisory committee whose members are all patients, caregivers, and representatives of patient organizations. The FDA established the committee to help assure that the needs and experiences of patients are included as part of the FDA's deliberations on complex issues involving the regulation of medical devices and their use by patients. This committee brings patients, caregivers, patient organizations, and experts together for a broader discussion of important patient-related issues.

During the inaugural meeting of the PEAC held in October of 2017, the committee discussed and made recommendations to FDA on patient engagement and medical device clinical studies and the role of patient advisors in designing clinical investigations, from recruiting, enrolling, and retaining study research participations in clinical studies, and identifying opportunities and barriers patient advisors face when collaborating with industry in the clinical study process. In a consensus recommendation, the PEAC stated that a framework should be developed to clarify how patient advisors can more effectively engage in the clinical study process.

FDA released a discussion document to facilitate further public discourse on patient engagement and medical device clinical trials. The discussion document described FDA's initial thoughts about patient engagement and its potential impact on medical device clinical studies. The discussion document included targeted questions on which the agency sought public feedback through an open public docket. This was shared at the second PEAC meeting in November of 2018.

FDA co-sponsored a public workshop with the Clinical Trials Transformation Initiative, also known as CTTI, in March of 2019. During this workshop stakeholders, including patients, industry, and key opinion leaders in clinical research discussed best practices and key considerations for enhancing the incorporation of patient perspectives on clinical trial access, design, conduct, and post-trial follow-up.

Based on the PEAC recommendations, the public feedback on the discussion document and the dialogue at the CTTI workshop, FDA developed this guidance document. The draft guidance was published in September of 2019, prior to this final guidance, which was issued on January 26 of this year.

Now let's cover the meaning of patient engagement and relative definitions in the context of this guidance.

For purposes of this guidance, patient engagement is defined as intentional, meaningful interactions with patients that provide opportunities for mutual learning and effective collaborations. Patient engagement in the context of planning for a clinical study creates opportunities to consider patient experiences, needs, and priorities in study design and conduct.

In this guidance, FDA draws on an important distinction between this type of patient engagement and the interactions that sponsors or clinical researchers have with individuals who are enrolled in a study as study and/or research participants. Patients are defined as individuals with or at risk of a specific disease or health condition, whether or not they currently receive any therapy to prevent or treat that disease or condition. They are individuals who directly experience the benefits and harms associated with medical products. This may include patients who are healthy individuals who may be undergoing screening or diagnostic tests, or individuals living with a medical condition and interfacing with medical devices to treat the specific disease or health condition.

For the purposes of this guidance, study or research participants refers to individuals who are or become a participant in research as a recipient of the test article on whom or on whose specimen the test article is used, or as a control, and may include healthy individuals. FDA regulations use the terms "subject" or "human subject" to refer to these individuals, but patients may be familiar with a different term. Therefore in this guidance, the term "study or research participant" is used instead.

For purposes of this guidance, the term "patient advisors" refers to individuals who have experience living with a disease or condition and can serve in an advisory or consultative capacity to improve clinical study design and conduct, but who are not study research participants themselves or caregivers of study or research participants. Similar to clinical advisors and experienced clinical researchers, patient advisors may provide recommendations that positively impact how a study is designed and conducted, improve the patient experience during this study, and improve the relevance, quality, and impact of study results. To avoid potential real or perceived conflicts of interest, these patient advisors should not be study or research participants in the same study for which they are advising. An allowable exception may arise in the case of clinical and studies involving rare diseases. In these cases, the guidance allows for case-by-case discussion with appropriate review divisions.

Patient advisors may have participated in previous clinical studies of the same disease or condition or similar device type. They may have been screened for but ultimately did not qualify for or did not elect to participate in a similar clinical study. They may be representatives from a disease-specific or cross-cutting patient organization. They may be healthy individuals who may be potential diagnostic device users or caregivers of patients who may have experience with the disease, condition, or device. For example, a clinical study being designed to evaluate the performance of a mammography device might enlist women who have experienced mammograms, regardless of whether they have a particular medical diagnosis. These women may be patient advisors.

FDA received feedback from patients and industry at the 2017 PEAC meeting and a public docket indicating broad support for patient engagement in clinical studies. However, we heard about several common perceived barriers and challenges to such engagement, including but not limited to the perception that patient engagement in the design and conduct of clinical studies is not allowed by FDA or valued by research teams, or challenges finding patient advisors knowledgeable about clinical study methodology, and site investigators' reluctance to allow sponsors to engage with patients except as study or research participants, and the logistical challenges of engaging with patient advisors in person, which may preclude their involvement in the design of clinical studies, and also challenges with determining which patient advisors or patient organizations should be engaged, and if multiple advisors are engaged, how to reconcile the differing perspectives. Similar comments were expressed at the 2019 FDA CTTI Public Workshop. This guidance intends to address some of these perceived barriers and challenges.

Now I'll give an overview of the guidance.

The purpose of this guidance is to help sponsors understand how they can voluntarily use patient engagement to elicit experience, perspectives, or other relevant information from patient advisors to improve the design and conduct of medical device clinical studies, highlights the benefits of engaging with patient advisors early in the medical device development process, illustrate which patient engagement activities are generally not considered by FDA to constitute research or an activity subject to FDA's regulations, including regulations regarding Institutional Review Boards or IRBs, and address

common questions and misconceptions about collecting and submitting to FDA patient engagement information regarding the design and conduct of a medical device clinical study.

The structure of the guidance begins with an introduction and specification of guidance objectives, including FDA's views on the value that patient and caregiver perspectives about their disease condition and the impact of medical devices brings to clinical study design. The background describes activities leading up to the guidance issuance, outlines numerous possible benefits of input from diverse patient advisors, and highlights perceived barriers and challenges to patient engagement in clinical studies. This is followed by scope then definition of patient engagement and the two distinct roles for patients that we just reviewed. Then we have questions and answers on patient engagement clinical studies, and ways that industry might engage with patients, ultimately leading to greater efficiency and quality of medical device clinical studies and greater uptake of results by patients and providers when making treatment decisions about legally marketed medical devices and earlier U.S. patient access to beneficial medical devices.

This guidance focuses on application of patient engagement by using patient advisors to inform and improve the design and conduct of medical device clinical studies. This guidance does not address study or research participant or patient advisor reimbursement or compensation, promotion of investigational devices, or dissemination of clinical study results.

Successful adoption of legally marketed medical devices increasingly depends on patient acceptance of that technology and patients being more engaged in the health care process along with demonstrated public health benefits. FDA believes effective patient engagement can help mitigate some of the practical challenges to robust clinical studies, including challenges concerning study and research participant enrollment and retention in the study, particularly when protocols include lengthier follow-up periods, like two years post procedure, and/or frequent visits to the clinical site, which may require significant travel.

Additionally, study plans for medical device studies may be complex, with many endpoints as well as eligibility criteria that exclude some study and research participants living with a disease or condition from participating in clinical studies. When not adequately addressed, each of these factors can contribute to increased time and cost to study sponsors, increase burden to study and research participants in the health care system, and delays in U.S. patient access to beneficial medical technologies.

FDA believes medical device clinical studies prospectively designed with input from diverse patient advisors, including those from racially and ethnically diverse populations, may help to address common challenges faced in these clinical studies and could result in the benefits outlined on this slide. Such as faster study, research participant recruitment, enrollment, and study completion, greater study or research participant commitment and retention resulting in decrease loss to follow-up, greater study or research participant adherence resulting in fewer protocol deviations or violations, fewer protocol revisions, streamlined data collection resulting in better quality data, and more relevant data on outcomes that matter to patients.

Now that we've talked about the guidance, let's talk about how you can apply patient engagement in your study planning activities.

So when can patient advisors be involved? Sponsors should consider involving patient advisors during the early planning phases of the clinical study so that their input can be incorporated while the study plan is being developed, especially in innovative areas or new target patient populations. We encourage sponsors to confer with patient advisors when designing or planning the clinical study. This could help identify which potential endpoints are meaningful to patients and avoid unanticipated issues that could hinder recruitment.

For ongoing studies that face significant challenges with study or research participant recruitment and/or retention, sponsors may want to consider involving patient advisors along with the study coordinator to troubleshoot and propose potential solutions. Sponsors may also consider involving patient advisors post-study to inform improvements for future studies. In more established areas, patient advisor input on draft study plans may make the design more patient-centric, while also translating to time and cost savings. Such input should generally be incorporated before the final protocol and informed consent documents are submitted to the IRB for review or to FDA, if an IDE application is required.

Some patient engagement activities that may enhance the design and conduct of clinical studies include but are not limited to improving the informed consent document to ensure patients understand the information presented for the clinical study, obtaining input on flexible options for follow-up visits and data collection techniques to reduce unnecessary burden on study or research participants who may have challenges fulfilling the follow-up schedule, reducing recruitment barriers and/or issues, such as causes of study delays or challenges not anticipated before the study, discussing which potential endpoints are meaningful in the treatment of the specific disease or condition, identifying potential barriers to participation, particularly those from under-representative groups, and ways to improve recruitment, challenges, or other experiences during the study to help streamline and improve future studies, informing the concepts that should be captured by patient reported outcome measures in the clinical study to better reflect outcomes that are important to patients, and informing the design of patient preference studies to inform the development of clinical studies or to help understand the benefit-risk trade-offs among patients for the proposed treatment or multiple treatment options used for the disease condition.

The primary purpose of the IRB review is to assure the protection of the rights and welfare of humans participating as study research participants. Because patient engagement activities with patient advisors primarily involve interaction in a consultative or advisory capacity, FDA does not generally consider patient engagement activities with patient advisors to constitute research or an activity subject to FDA's regulations. Therefore FDA's research regulations, including IRB requirements, generally would not apply.

In contrast, interactions between study or research participants and investigators typically include collecting information as part of a research plan that outlines the methodological approaches to be used. Such interactions are generally in the context of a clinical investigation subject to FDA's regulations and must satisfy the applicable requirements.

Now we'll go over some resources on patient engagement.

FDA is here to support you. We would like to engage you early in the planning process. To request feedback from FDA on your patient engagement plan or patient-centered study design, sponsors are encouraged to use the Q-Submission process. We encourage sponsors to reference any previous patient

engagement activities used to inform the development of the study plan. Sponsors may also use and cite relevant information from their patient engagement activities in their subsequent marketing applications to FDA.

In summary, FDA encourages patient engagement in medical device clinical studies in appropriate circumstances. This guidance document provides an overview of the potential value as well as a summary of the perceived challenges and some potential solutions related to involving patient advisors in the design and conduct of clinical studies. This document also identifies a variety of ways sponsors may engage patient advisors to design more patient-centric studies that may be more likely to enroll and retain study or research participants as well as collect information that's meaningful to patients.

If you are considering incorporating input from patient advisors in the design or conduct of your medical device clinical study, you are encouraged to engage in early interactions with FDA and obtain feedback from the relevant FDA office or division on appropriate design and any applicable regulatory requirements. FDA believes appropriate patient engagement may lead to improved efficiency and quality in the design and conduct of medical device clinical studies, and greater uptake of results by patients and providers when making treatment decisions about a legally marketed medical device, ultimately advancing U.S. patient access to beneficial medical devices.

**CDR Kimberly Piermatteo:** Thank you, Tracy, for that great overview. Now let's transition to the interactive question and answer segment of our program today.

Joining Tracy today for this segment is Katherine Capanna, Deputy Director in the Division of All Hazards Response, Science, and Strategic Partnerships, or DARSS, in CDRH's Office of Strategic Partnerships and Technology Innovation, or OST; Mimi Nguyen, Regulatory Health Project Manager, also in DARSS in OST; and Dr. Allen Chen, Program Manager in DARSS as well in OST.

Before we begin, I'd like to go over a few reminders about this segment. To ask a question, please click the Raise Hand button, which should appear on the bottom of your Zoom screen. I'll announce your name, unmute your line, and invite you to ask your question. You'll receive a prompt to talk on your Zoom screen. Please acknowledge this prompt and then ask your question.

After you ask your question, please lower your hand. If you have another question, you may raise your hand again to get back into the queue and we will call on you again later if we have time. Also, when asking a question today, please limit yourself to one question only and try to keep it as short as possible. Please also refrain from asking about specific submissions. For these questions, we do ask that you consider submitting a Q-Submission or consider emailing my division at [DICE@fda.hhs.gov](mailto:DICE@fda.hhs.gov).

Now, as we wait to receive some of your questions, I'd like to welcome our newest panelists with a few questions that we've gotten over the past few weeks about the guidance. The first question I will direct towards you, Katie, and that question is, how does this guidance further the FDA's efforts around patient science and engagement?

**Katie Capanna:** Good afternoon. And thank you, Kim, and thanks to everyone who has joined us this afternoon. This is an important milestone for us in our program for patient science and engagement here at FDA Center for Devices and Radiological Health.

The Patient Science and Engagement Program, the goal of that program is to proactively integrate patient perspectives on living with their health condition as well as its treatment or management, and to do so across the total product life cycle for medical devices to help promote and protect public health. And clinical studies are an important source of learning and scientific evidence on how medical devices perform in the intended patient population.

So incorporating patient perspectives can help improve clinical study design and conduct, and this guidance that Tracy overviewed will help stakeholders understand how they can tap into patient advisors to help make studies more patient-centric. By doing so, this can improve the efficiency of recruitment and study completion, can save time and cost for the clinical study, and can ultimately improve the relevance, the quality, and the impact of study results.

**CDR Kimberly Piermatteo:** Thank you, Katie. Alright, our next question that we received previously, well, I'll direct that one to you, Mimi. And that question is, where can I learn more about best practices in patient engagement? Does FDA have a list of resources?

**Mimi Nguyen:** Thank you for the question, Kim. FDA has a webpage on patient engagement. You can Google search CDRH patient engagement to find our webpage that shows an overview of the ways we engage with patients and encourage sponsors to do so as well. This page includes links to our guidances and other program documents related to patient science and engagement that will be helpful.

In addition, FDA cannot necessarily endorse the work of outside organizations, but we have collaborated and worked with other stakeholders and have shared some of their work at other FDA meetings, like the Patient Engagement Advisory Committee and other public venues. Some of these have included works on methodologies and best practices for increasing patient centricity in the health care system, for example, some of the public-private partnerships, such as CTTI that was mentioned earlier, the Clinical Trials Transformation Initiative, as well as the Medical Device Innovation Consortium, or MDIC. Other organizations also have some resources, such as PCORI, which is the Patient-Centered Outcomes Research Institute; ISPOR, the Professional Society for Health Economics and Outcomes Research; and the National Health Council, or NHC.

For best practices in patient engagement, stakeholders should also look to some of our collaboration efforts that we've had. In 2019, the FDA and CTTI did a public meeting talking about patient engagement, which was referenced earlier in the presentation and actually is linked to in the guidance document itself.

**CDR Kimberly Piermatteo:** Thank you, Mimi. Alright, this next question I'll direct towards you, Allen. The question is, the guidance notes that patient engagement could help address common challenges faced in clinical studies. Could you please elaborate on this?

**Allen Chen:** Sure. Thank you for the question, Kim, and good afternoon. I'd be happy to highlight a few examples to elaborate on where the guidance notes this. First, recruitment and retention of patients is one of the most common challenges. Many clinical studies struggle to recruit, enroll, and retain patients in a timely way.

This could be partly attributable to aspects of the study design that could be modified to be more patient friendly. For example, input from patient advisors could help sponsors identify more flexible options for follow-up visits and data collection techniques, such as allowing extended or weekend hours,

permitting local clinicians to perform some follow-up assessments, or using mobile or online technologies to enable virtual or remote follow-up. When studies are underway, sponsors can also work with patient advisors on an ongoing basis to identify ways to alleviate barriers and challenges that may be contributing to lower recruitment or high cost to follow-up.

Second, many study sponsors may find value in applying patient advisor input to improve the informed consent document to ensure better comprehension of the information about the study. And finally, particularly for novel innovation areas, such as potential breakthrough devices, FDA encourages sponsors to consider how patient input could help inform which potential endpoints are meaningful to patients with a specific disease or condition in their study. Back to you, Kim.

**CDR Kimberly Piermatteo:** Thank you, Allen. Alright, now we will go ahead and move to take your live questions. So our first question from the audience looks like it's coming from Shahan. Shahan, I'm unmuting your line. Please make sure you unmute yourself and ask your question. Shahan, are you still there?

**Shahan Stephanian:** OK, I'm here. Sorry. Well, my question is the outcome of the patient engagement as an input, will it make any significant changes in the user needs and the user requirements and then marketing requirements from the design perspective? So how that will be combined, in terms of the information and translated to design input, for example, design control requirements, that's my question.

**CDR Kimberly Piermatteo:** Thank you for that question. Katie, would you like to provide a response?

**Katie Capanna:** Sure. I'm happy to take that, and thank you for the question. Let me just paraphrase to make sure I understood. Are you asking about what companies should do if they receive patient input that suggests changes that might be beneficial, that would impact design of the device itself? Am I understanding your question correctly?

**Shahan Stephanian:** Yes, that's correct. Yes.

**Katie Capanna:** OK, wonderful. So CDRH certainly encourages including patient perspectives in this way. That is somewhat outside of the scope of this guidance, which is focusing on patient engagement to inform the clinical study design itself. But if you have a product area where you are incorporating patient perspectives and it is impacting your use of requirements in a way that you think will affect your device design, you are more than welcome to reach out to us to have a conversation about that and see if we can address the specifics of your case on a case-by-case basis.

**Shahan Stephanian:** Thank you.

**CDR Kimberly Piermatteo:** Thank you, Katie. Alright, our next question is coming from Vaishali. Vaishali, I'm going to unmute your line. Please unmute yourself and ask your question. Vaishali, are you able to unmute?

**Vaishali Patel:** Hi, can you hear me?

**CDR Kimberly Piermatteo:** Yes, I can.

**Vaishali Patel:** OK, great. Thank you so much. Thank you for the presentation and for a clear and concise guidance. I'm new to the device industry, so this question, I'm sure, have has been asked before. Since this guidance is published by CDRH, but it does seem like something that could be applicable for CDER and CBER, my question is, is it a guidance that can be used for other divisions of FDA as well?

**CDR Kimberly Piermatteo:** Thank you for that question. I'll go ahead and direct that question to Allen.

**Allen Chen:** Thank you for the question. So what I can say is that CDRH, CBER, and CDER, we all coordinate and collaborate on our various patient science engagement efforts. However, this specific guidance was inspired by our first Patient Engagement Advisory Committee, or PEAC, meeting, which was focused on medical devices. At this time the guidance only addresses medical devices regulated by CDRH and CBER.

**CDR Kimberly Piermatteo:** Thank you, Allen.

**Vaishali Patel:** Thank you.

**Allen Chen:** You're welcome.

**CDR Kimberly Piermatteo:** Alright, our next question is coming from Cher. Cher, I'm going to unmute your line. Please unmute yourself and ask your question.

**Cher Thomas:** As a patient advocate, I was curious how patients can volunteer, or are they appointed to become a patient advisor?

**CDR Kimberly Piermatteo:** OK. Thank you for that question. Tracy, would you like to take a first stab at that question?

**Tracy Gray:** Sure. So essentially, sponsors are encouraged to work with patient advisors by looking for those organizations who would have patients that would be able to contribute in a positive way. Those patients may have had experience with a medical condition or device, or they may be patients who have participated in other studies and have experience in that way. And so those patients would be able to bring great insight to and perspective to the clinical study design.

So really looking at different patient organizations, what their area of focus is, and you can Google that, looking at other studies that currently exist, and seeing the types of patients or patients who may have had prior experience but would not be able to participate in a dual role. They could not be in another study design at the same time in addition to participating as an advisor. So their prior experience may help them to be qualified. Does that answer your question?

**Cher Thomas:** I believe it does. So what you're saying is, for instance, if somebody wanted to find-- if a patient wanted to participate, what they would need to do is Google the opportunities that are available. Correct? And then put in a submission?

**Tracy Gray:** So when you're saying specifically for the sponsor, that-- I think I heard somebody else trying to jump in. But if you're really talking about specifically a sponsor, I don't know that it would be just an interaction with them. But if you do see opportunities, yes. And if you know somebody within your organization that has had the types of experiences that they're looking for and that would be able

to contribute, often they're looking for people too that may have had prior experience, that would be able to share that type of perspective.

**Katie Capanna:** This is Katie. I just wanted to add on to what Tracy said. And thank you, Cher, for the question. This is actually a question that we have been getting increasingly, both from patients who want to participate as well as from medical device study sponsors who are wondering, how can I find patients who have the condition of interest and may have experience that they would want to share in this kind of a capacity.

And so right now, we are not aware of any sort of centralized hub, so to speak, for identifying patients who are interested in serving as patient advisors on medical device clinical studies, but it is, as I mentioned, an increasingly common question, so we will be looking into that. I think in the interim, there are some of the resources that my colleagues have mentioned, in particular the Clinical Trials Transformation Initiative, who have done some work in the past on how patient groups can be partnered with by clinical trial sponsors over the course of the clinical trial design and conduct. And so I would encourage folks from patient advocates as well as study sponsors to keep an eye on the CTTI website for anything that might be relevant to that question and to your interest.

**Cher Thomas:** OK. And thank you, everybody on the panel. I appreciate it. I am a patient advocate with the Renal Support Network. And obviously, within our organization and the patients that interact with us, we have a large number of patients that have utilized different types of dialysis treatments, and so just a little-- in case anybody's looking for patients, we certainly have a large contact with that patient demographic.

**Katie Capanna:** Oh, that's wonderful. Thank you. Maybe we can-- if you wouldn't mind contacting us at the central mailbox that was displayed during the presentation, we would be very interested in following up with you after this call. Thank you.

**Cher Thomas:** Super. Thank you very much.

**CDR Kimberly Piermatteo:** Great. Thank you, Cher. Thank you, Tracy, and thank you, Katie. Our next question comes from Daniel. Daniel, I'm unmuting your line. Please unmute yourself and ask your question.

**Daniel Amin:** Thank you for the presentation. Can you hear me?

**CDR Kimberly Piermatteo:** Yes, we can.

**Daniel Amin:** OK. Now, I think my question is a follow-up to what we have been discussing so far. Let's assume that a company has come up with a new product and they want to conduct a clinical trial, and they know someone who, like say a celebrity, who has had experience with that disease. Are there measures in this guidance that could prevent the celebrity from promoting this product, which may influence the informed consent process? Because if I understood clearly, the IRB is not involved at the time of interaction between the patient advisor and the company.

So what can stop-- what are the measures in place to prevent any interference at the level of informed consent process? In other words, how can we avoid someone influencing another to participate in a study? Thank you.

**CDR Kimberly Piermatteo:** Thank you for that question. I'll direct that one to Katie, if you'd like to provide a response.

**Katie Capanna:** Yes. Thank you for that question. I think this issue that you're raising is certainly one that the agency has heard before, whether it's the informed consent process or other aspects of communication while a study is ongoing that could potentially impact, influence, bias, or otherwise jeopardize the impartiality of the study conduct. I think that's something that we don't specifically address in terms of the guidance policy here that we're discussing today, but would be something that would need to be carefully planned for in terms of the study sponsor and the engagement or participation or partnership that you all would be considering with. Whether it's a celebrity patient or a non-celebrity patient, I think those terms of engagement may be something that the sponsors would want to think about in advance.

**CDR Kimberly Piermatteo:** Thank you, Katie. Alright, our next question is coming from Brian. Brian, I'm unmuting your line. Please unmute yourself and ask your question.

Brian, we still can't hear you. If you can unmute your line, and then ask your question.

Alright, Brian we can't hear you. I will go ahead and lower your hand if you still have a question, please just raise your hand again and we will try to come back to you later on.

Alright. Our next question, then, is coming from Weiyang Zhao. I am going to unmute your line. Please unmute yourself and ask your question.

**Weiyang Zhao:** Hi. Can you hear me well?

**CDR Kimberly Piermatteo:** We can hear you.

**Weiyang Zhao:** OK, great. So my question is, during an IDE submission, are sponsors required to disclose if they have used a patient advisor in the submission? And if so and to which extent how much details are the sponsor need to put in the IDE submission about the patient advisors' inputs, et cetera?

**CDR Kimberly Piermatteo:** Thank you for that question. Tracy, are you able to answer that question? Or I think Katie may want to comment.

**Tracy Gray:** Yes, I can start out. So one thing that we are very happy-- thank you, first of all, for your question, and we are happy to address inquiries that come from sponsors. And one thing is we do encourage early feedback and interaction with FDA as you're developing plans for breakthrough devices or other innovative areas that might benefit from having patient input early. That would help to shape the clinical study design, things like helping with the informed endpoint selection. So we do encourage that if it's for a breakthrough device or innovative area, to do that sooner than later.

And then we also know that sponsors may seek feedback if there are protocol changes that maybe a patient advisor has recommended. And for that type of feedback, you would go ahead and include that with your Q-Submission, and also note in your cover letter that you do have a patient input request included in your submission, and that way it'll flag it for us internally. Katie, or did anyone else have something they wanted to add on to that?

**Mimi Nguyen:** This is Mimi. Yeah, I think Tracy has mostly covered it. Everything is not required, but we do encourage you to share with us if you've had input from a patient advisor, especially, as Tracy was saying, if there was some documented changes based on the input. It's helpful for us to understand why those changes were made. Thank you.

**Weiyang Zhao:** Thank you.

**CDR Kimberly Piermatteo:** Thank you, Mimi and Tracy. Our next question is coming from John. John, I have unmuted your line. Please unmute yourself and ask your question.

**John Rice:** OK. Am I heard?

**CDR Kimberly Piermatteo:** I can hear you, yes.

**John Rice:** OK. And I want to go back a little bit to that how do people become connected that are really interested in contributing as a patient advisor. And has the FDA considered maintaining, in its simplest form, a mailing list where those of us who are interested could put our names so that potential sponsors have a way to connect and say that they're looking for people, or a little more sophisticated, maybe, where we would answer some basic questions in that database. Because one of the problems I see where you're really trying to get sponsors to involve patients early, there's some proprietary-ness in what a sponsor may be doing where they don't want to go out and advertise something that they're working on to the whole world yet, but they still need a way that they can find patient advisors. And so some kind of a database where there's just enough in it to help the sponsor identify people that have volunteered, so is that something that the FDA may be able to set up?

**CDR Kimberly Piermatteo:** Thank you, John, for that question. Tracy, would you like to answer that?

**Tracy Gray:** Hi, John. Thank you so much for participating in the webinar and for your question. So unfortunately, we don't have any kind of a database at this time or a list where we actually track patients who have an interest, but that certainly is great feedback for us to consider. And we appreciate your thoughts about that and your desire to be able to find opportunities.

One thing that Katie had mentioned earlier was there are stakeholders out there who, and patient advocacy organizations, who often have an arm of patients within their organization who have specific medical device conditions and may have participated in trials before. And there are also ways that you can search for clinical studies that are underway, but you're probably interested in the groundwork, like being able for sponsors to identify early on which patients may be able to provide input.

**John Rice:** Right.

**Tracy Gray:** One thing that came up during the 2017 Patient Engagement Advisory Committee and other public forums is that recommendations were made on making sure that there was diverse patient input in clinical investigations, and so one suggestion was partnering with patient groups, physicians, or sites and organizations to leverage their networks. So you may find through those connections that there are opportunities, and also having knowledge about how to reach diverse patients and patients that may have certain types of experience.

Some examples in the literature suggest that involving investigators from underrepresented groups can help with recruitment of populations in the study, and physicians may be partners in helping us to identify diverse patient advisors. Several resources that point to the importance of considering geographic location. For example, as we're looking for a variety of patients and with diverse views, I'm looking at sites that may be urban or rural or that have a high concentration of populations that are historically underrepresented in clinical research to help us find patients with diverse patients.

And then also strengthening strategic partnerships with other patient groups, physicians, and organizations, like those mentioned before, the Patient-Centered Outcomes Research Institute, which has a conceptual framework for recommendations for engaging diverse populations and engaging strategies to achieve principles like trust, reciprocal relationships, transparency, and cultural competency.

In addition, CDRH also is advancing health equity as one of its strategic priorities. And as part of this work, we will take steps to reduce barriers to increase opportunities for participation by diverse populations in clinical trials. So we just ask that you stay tuned for that. So while we don't have a database at this time, hopefully some of the information that I shared will give you some things to consider as possibilities. Does anyone else have something they'd like to add?

**John Rice:** If FDA would like a couple of patient advisors for the advisory program, perhaps there are some people who would want to help develop that mechanistic system that makes it easy for volunteers and for sponsors to get connected, and I'd love to participate in something like that.

**Tracy Gray:** Well, thank you so much, John. We really appreciate that, and we appreciate your input during this webinar and for your recommendations.

**CDR Kimberly Piermatteo:** Thank you, Tracy.

**Allen Chen:** Thank you very much, John.

**CDR Kimberly Piermatteo:** Yeah. Thank you. Alright, we have time for one more question. Brian, I'm going to try to unmute you again. Please unmute yourself and ask your question.

**Brian Jones:** OK, is it working now?

**CDR Kimberly Piermatteo:** Yes, we can hear you.

**Brian Jones:** Thank you. It is more clear to me how to approach groups who have already been identified to have a disease and to seek their perspective. But it's less clear how to identify appropriate patient advisors or advocacy groups for devices that are intended for screening average risk populations. I worry about seeking patient groups who have been identified with the disease because their perspective may be different from a screening population. So is there any additional advice FDA could provide on that?

**CDR Kimberly Piermatteo:** Sure. Thank you for that question, Brian. I'd like to go ahead-- Tracy or Katie, would you like to take that question?

**Katie Capanna:** Sure. That's an excellent question. I can certainly understand the concerns that you articulated there. If you have a specific study or technology in mind, what I would suggest is that you

reach out to the patient engagement email address that was shared by Tracy earlier so we can discuss the specifics of that particular case.

**Brain Jones:** OK. Thank you.

**CDR Kimberly Piermatteo:** Alright, thank you, Katie. That wraps up our live Q&A questions. We appreciate everyone's very engaging discussion. So at this time, I'd like to go ahead and turn it over to Tracy for her final thoughts.

**Tracy Gray:** Thank you so much for that, Kim. And thank you, everyone, for joining our webinar today. I just want to reiterate that patients and caregivers can bring valuable expertise and perspectives about a disease or condition and help improve clinical study design and conduct by serving as patient advisors.

If you are considering incorporating input from patient advisors in the design or conduct of your medical device clinical study, we encourage you to engage FDA early on appropriate design and any applicable regulatory requirements. More patient-centric medical device clinical studies may lead to improved efficiency and quality of clinical study design, leading to earlier U.S. patient access to beneficial medical devices. Thank you so much, everyone.

**CDR Kimberly Piermatteo:** Thank you, Tracy, for those final thoughts and for your presentation today on this final guidance. I'd also like to, again, thank our panelists, Katie Capanna, Mimi Nguyen, and Dr. Allen Chen, for their discussion today. And thank you to all of you, our audience, for your participation today and the questions.

So printable slides for today's presentation are currently available on CDRH Learn at the link provided on this slide, under Specialty Technical Topics, specifically the subsection Patient Engagement. A recording of today's webinar and transcript will be posted on CDRH Learn in a few weeks. That link is also provided on this-- or the screenshot is provided on the slide where you can find those presentation materials.

For additional questions about today's presentation, you may email us at [DICE@fda.hhs.gov](mailto:DICE@fda.hhs.gov). We also encourage you to attend a future CDRH webinar. The link on the bottom of this slide provides a listing of all of our scheduled upcoming webinars.

And with that, this concludes today's CDRH webinar. Again, thank you for joining us today, and have a nice day.

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