

TRANSCRIPT: Virtual Trials Bring Research to Patients

STEPHEN BALEVIC: So when we think about a traditional clinical trial, this means you know, most of our patient engagement is happening at a site, so that means either something like a clinic or a hospital. And that's been our clinical trial, you know paradigm, you know, for the last several decades. One of the challenges, though, is that it's very, very hard to conduct that type of research with rare diseases like what we encounter as pediatric rheumatologists—in particular, it places actually fairly high burden on patients and their families.

So in the virtual trial model, we sort of turned that traditional paradigm on its head—and so, instead of patients and their families coming to us to participate in research, we go out to them. And so, in the incompletely virtual trial model, all the patient engagement, all the study visits happen outside of a brick and mortar building and actually in the patient's home.

And so this does a number of things—one, it makes it much, much easier to engage patients and their families; it reduces the burden for them to be able to participate. But also, we can reach a much wider patient community, so we have a much more diverse research population, and we have results that actually reflect the patients that are out there.

VINCENT DEL GAIZO: From my perspective, that is one of the main barriers to participate in research is just logistics. Like you said, chances are you live far from the clinic, you have work, your child has school, you know, and driving back and forth for these for these research-related visits are definitely a barrier for families, there's no question.

CARRA made an investment, a commitment to patient engagement. We have families that actually help docs on the other side of the curtain. They partner with the researchers like Dr. Balevic to design studies that are more acceptable to families, to help communicate what the study is about, why it's important from the patient perspective. So now we're designing studies that are more acceptable and have greater impact. Families are helping name the study, select additional secondary outcomes of studies, so on and so forth.

And that's where kind of I come in and my role is—when Stephen approached me about iPERSONAL and said hey, he wants families to help him, then I turn to the community and find some families that would be kind of the target audience of participation in the study and recruit them to research partners, train them to be research partners. Because it is very overwhelming for families—they need to be empowered, they need to understand what their roles are, and then explain the study to them and then bring everybody together so that the study team and the families are all meeting to talk about the study, make modifications to the protocol, if necessary, to make it more acceptable, and then, once we're done, share it with the community.

STEPHEN BALEVIC: The CARRA Registry was really a great partnership for a number of reasons, you know, for the iPERSONAL study. The parent and patient stakeholder advisory group that we developed was really critical in helping to answer a lot of not only logistical questions, but in helping us sort of design the protocol. You know, so this is the very first trial we've conducted in pediatrics using this fully virtual trial design, so we had a lot of questions, right, so how do patients and their families feel about a

stranger, in this case, a research coordinator, coming into their home. So I think the CARRA partnership for that patient engagement piece was huge.

VINCENT DEL GAIZO: Really, we want all studies to consider having patient partners help them with designing the study and all the way out to sharing the results of the study. Because that's a really important part of the work that we're doing, is when the results are ready, they get published in some medical journal in language that families can't understand.

So how do we get those results back to the community so that they can use them, they can get the information that they desperately need to make important decisions on their care for their desired outcomes.