Pariser, Anne 2021

Dr. Anne Pariser Oral History

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Dr. Anne Pariser

Behind The Mask

June 22, 2021

Barr: Good morning. Today is June 22, 2021. My name is Gabrielle Barr, and I'm the archivist with the Office of NIH History and Stetten Museum. Today I have the pleasure of speaking with Dr. Anne Pariser. Dr. Pariser is the director of the Office of Rare Diseases Research at the National Center for Advancing Translational Sciences, and today she is going to speak about some of her COVID work and research. Thank you for being with me.

Pariser: Yes, thank you for having me.

Barr: Can you introduce the rare diseases clinical research network that NIH supports, who is a part of it and what type of mission it has?

Pariser: The Rare Disease Clinical Research Network (RDCRN) is a network of Centers of Excellence, called "consortia", for rare diseases. Through these consortia researchers, clinicians, patient groups, and other multi-disciplinary stakeholders work together to study rare diseases. One requirement of the program, and what makes the RDCRN unique, is that researchers are required to study three or more rare diseases at the same time, and they need to be related somehow. There's a great deal of flexibility in how they're related. For example, it could be around an organ—like the Rare Lung [Diseases] Consortium—around an organelle like a mitochondria—some of these are mutationally driven, or any other collaboration that the investigators propose.

The theory here is that we have many rare diseases. We have between seven and ten thousand rare diseases that we know of. Studying them one disease at a time is too slow, is going to take too long, and we have too many unmet needs. So, [with] this collaborative approach, we're trying to include more rare diseases, but also trying to learn from each other, either within a consortium or even across the network. To be part of the network, investigators agreed to study three or more diseases at the same time and work collaboratively. Patients are integrated into leadership roles as part of the research process. But also, there are requirements to share knowledge, data, and experiences. We focus right now in this iteration on clinical trial readiness—what are the steps we need to get in the clinic for therapeutics if we can?—and data standards and practices to enable information sharing. This is a program that's been up and running since 2003. We're now in the fourth 5-year awardee cohort for the network. Currently we have 20 consortia and they're studying about 250 diseases through this model. Each consortium has a principal investigator located at an academic institution in the U.S., but they're multi-site, and they collaborate, so we have about 150 or so different clinical sites, most of them are at academic centers.

Barr: All over the world or primarily in the United States?

Pariser: Primarily in the U.S. but there are international collaborators, predominantly in Europe and Canada. We also include about 150 Patient Advocacy Groups—or PAGS—as part of the network for these diseases.

Barr: Wow, that's very interesting. When did you begin developing the survey that looks at how COVID-19 impacts those individuals with rare diseases and their families as well as their caregivers?

Pariser: This actually came out of the network itself. The RDCRN consortia, these Centers of Excellence I told you about, are all held together by a Data Management and Coordinating Center—the DMCC—which is currently centered at Cincinnati Children's Hospital. What a number of the investigators were hearing from their patients is that they were having trouble accessing care. They sometimes couldn't get to the therapies they needed. Many of our patients are very sick; they were feeling very isolated. They started hearing this from a number of different places. The DMCC, with some of the consortia, got together and decided what we really needed to do is put some data around this. Very quickly leveraging the network, they were able to put together a patient survey and then get the usual things you need—IRB [Institutional Review Board] approval—and put it up on the website and then disseminate this. I think it launched about the middle of 2020, which is really pretty rapid if you consider everything that was unfolding. The survey was open between three to six months and got over 4,000 respondents.

Barr: Wow!

Pariser: Yeah. The preliminary results are actually up on the DMCC website. There's been what's called a "spotlight newsletter" by the RDCRN about it. A couple of our investigators, Dr. [Marc] Rothenberg and Dr. [Thomas] Ferkel, from two of the consortia, presented some of the preliminary results at Rare Disease Day at NIH in February. There's a lot of data analysis going on right now, trying to get a deeper dive look into some of the results.

Barr: Can you speak a little bit more in detail about the preparation of the survey, such as how you went about crafting questions? I know there are so many diseases, you probably couldn't do specific questions for diseases.

Pariser: It wasn't specific to any disease. It was really more about some of the things you would expect— the demographics. Who are you? Where do you live? Which disease have you been diagnosed with? Have you had COVID or not? Have you been tested or not? If you did have COVID, that opened up a number of other questions about how severe it was. Were you hospitalized? Do you feel there were complications related to rare disease? But then there were also a number of questions relating to psychosocial. Have you been able to get care? Have you had problems getting to the emergency room or hospital if you needed it? Have you had trouble accessing medications or research? Because so many of our rare diseases don't have approved therapies, patients are often very dependent on experimental agents or ongoing clinical trials. Did they have barriers there? These were really general questions. The questionnaire was actually opened up to the entire rare disease community, you didn't actually have to be a patient within the Rare Disease Clinical Research Network to take the questionnaire.

It was very collaborative, like so many of our things are—team science and collaboration. The patient leadership in the PAGs, known as the CPAG [Coalition of Patient Advocacy Groups], helped craft the questions. They networked with their members about some things people were experiencing. The researchers themselves had questions that they wanted to know, for example, relating to research or how sick the patients were getting, but also [about] what they were hearing from their patients. The Data Management Coordinating Center was also hearing some of this and so it was really a very collaborative effort. There was debate, discussion, and testing of some of the questions before it eventually launched.

Barr: About how many questions were on the survey, and did everybody see the same questions?

Pariser: The core questions were the same, but if you answered yes or no to certain parts, it would then open up other questions. I'm not sure how many questions. There were a few dozen, I think, within these different buckets.

Barr: Very manageable.

Pariser: For example, if heaven forbid you did get COVID, then that would open up a whole bunch more questions about the clinical course and how you were treated, so it may have taken a little bit longer. But if you were negative to a lot of these things, it was fairly quick to complete. What we heard is most people were completing the questionnaire within 15 minutes. Sometimes it took them a little bit longer.

Barr: What do you hope that the results of this preliminary survey will accomplish, both in the short term and in the long term?

Pariser: Well, I think we've already learned some things. [laughs] One of the first things you look at is demographic data, you know, who answered the survey and who participated. Because it was an online survey, we found out we were only reaching certain people. About 90 percent of the respondents identified as white. A high proportion of the survey respondents were people who were within the RDCRN, which I suppose is understandable because there was a lot of advertising. One of the first things that was very evident is we really need to come up with a new strategy to try to reach all rare disease patients. We are not going to be reaching everybody, and this seems to be particularly disproportionate to underserved communities. This is something we're looking at right now, and in fact, the RDCRN has stood up an equity, diversity, and inclusion work group to try to start exploring some of these issues already.

Another thing that we saw—and this is again expected, and I don't think this is unique to rare diseases—is that a lot of clinical studies, particularly observational studies, were put on hold or patients were not able to come in for whatever reason. That could be very understandable—if you have a severe underlying condition, going out during the height of COVID was potentially dangerous, so people were not coming in. This has always been an issue with rare diseases; our patients tend to have mobility issues or other issues where it's hard for them to travel. Because the diseases are rare, there may only be a couple of study sites and they can be geographically very remote. I've been long interested in remote capture and telemedicine and decentralized clinical trials, but this really shown a spotlight on how this has just become a "have to have." We really have to have these types of things so that research and patient monitoring and patient care can go on regardless of whether you are able to come into the study site or not—and may actually improve the data that we're getting when people don't have to travel long distances. A lot of our patients are children, and they're fatigued when they get there. We may not be getting them at their full capabilities when they do show up for some of this testing. We've also taken steps in that direction to try to come up with concepts about remote capture or validation that could enable the use of these technologies in clinical trials.

Barr: Can you talk about some of those ideas that you that you're coming up with about that?

Pariser: Sure. NCATS [National Center for Advancing Translational Sciences]—like all the other institutes and centers at NIH—we have a council, which is like our oversight board. Before we go forward with any kind of funding announcement, we have to go to our council to seek concurrence with an idea. We've already gone to our council—this is a public meeting—and presented the concept of telemedicine and remote capture for rare diseases, specifically through the Small Business Innovation Research Program. There're so many little tech companies involved in this that could really help, so that concept has already gone forward so we're working on that. I think this was slightly pre-pandemic but has moved forward during the pandemic: virtual reality. That's created essentially a platform, almost like a video game, where patients could, for example, do physical therapy, or therapy, or communicate with their team on this platform. That also went through the Small Business Innovation Research grants. That was completed and hopefully we'll be making awards around that as well. This is really something fairly cutting edge. For example, a patient would select an avatar so you could augment the reality for collecting things like movement – trying to more precision around that. We think that's pretty exciting.

Barr: That is exciting! That's really neat.

Pariser: Yeah. That's moving forward; hopefully those will be awarded pretty soon. One other thing that came out of this survey as well is—NIH had a COVID serosurvey, where they were collecting blood samples from people all over the United States. Through this RDCRN survey, they collaborated so that could patients who completed the survey could be contacted to see if they also wanted to participate in the serosurvey. Again, that was stood up and was ongoing as part of this whole NIH serosurvey. What I understand is—I haven't seen it, but some preliminary results from this are supposed to be published in Science and Translational Medicine Journal today.

Barr: Oh, that's exciting!

Pariser: We expect to get more information rolling out on all of these things, but that should be available soon.

Barr: What are some ways that you hope to diversify? You were saying that you were working to diversify the candidates that respond to your surveys. What were some of the approaches they were thinking of?

Pariser: Right now, we have a work group stood up for that, but we rely very heavily on social media and online platforms, which a lot of the groups do. A lot of common and rare disease groups do. It's a way for people to get together from their own home and communicate and share stories. But that can't be the only solution, so we're looking at what other people have done. For example, at NIH the CTSA [Clinical and Translational Science Awards] Program—they do have community engagement strategies. Can we leverage something like that? Can we learn from what they did going out into non-traditional areas? I don't know if you saw this but there was an article—I think it was in the New England Journal a year or two ago—about hypertension monitoring in barber shops. You went to where the people are! Reach people through church groups, community leaders, schools. Are there other ways we can get to people other than what we've been traditionally doing? These are all things that are currently being discussed. This isn't true just for rare diseases, although we certainly saw that this is definitely a problem, and we do need to do better.

Barr: How have those with rare diseases fared with COVID? Both with the disease and just in general with their different medical considerations.

Pariser: The preliminary evidence—and this is preliminary again—[is] a number of patients did report they had trouble accessing services. Many of these diseases are serious, so our patients are dependent on emergency rooms and hospitals. Emergency rooms for a while there were completely overwhelmed, and patients were being discouraged from coming in. Doctors' offices were closed or were going to alternate means at times. Patients did report there were barriers to receiving care, and in some cases, some of the medications. For example, hydroxychloroquine. For a while there the supply was under pressure as it was being looked at. There are a number of rare diseases that actually do depend on that so there was a concern there. Did this result in bad outcomes for patients? We don't have any data on that, but these were definitely concerns that came out through the survey.

The other one was on clinical trials. Clinical trials and clinical studies. Some were paused for a while. It wasn't just rare diseases, but for serious illnesses, this was very much a concern. Some of our observational studies, the natural history studies for example, there's missing data. These are things that are going to roll out over time, we're going to see how this affects it.

Another thing, and again this is not just rare diseases, but medical conferences. A lot were canceled early on, thinking that this wasn't going to go on as long as it did, and a lot were postponed and turned into virtual meetings. That's been about a year or year and a half. Where it seems to be a big problem is particularly with younger investigators and fellows and students because this is often a foot in the door of getting your posters and networking. Everybody made a really heroic effort to keep these things up and running. You do miss a little bit of that human interaction, particularly for the next generation of researchers. We hope that there aren't long-term impacts from that, but we're going to have to follow this carefully.

Barr: What are some follow-up questions that you hope to get more answers on for another iteration for the survey, if you're planning for that?

Pariser: We don't have another survey planned right at the moment. We really do want to know how this impacted people's health. What I forgot to mention is [the] isolation a lot of people felt. For example, some of these diseases impact the immune system or the lungs, and there are people who did not leave their house, for a long period of time. Again, this is true across the board. What is that going to do to our mental health? We're very concerned about that. You add that on top of what is already a chronic or isolating disease. We have some concerns, and we definitely want to learn more about that and make sure that our patients are being looked after. Again, are there other areas of study this is going to open up? Are there certain rare diseases that were either disproportionately impacted because of underlying conditions? Or the other way, potentially protective, because we know the host responses in any infectious disease start to come out—genetic susceptibilities, for example, or other underlying conditions. There's probably a lot to learn there that could benefit everyone.

Barr: Definitely. What were ways that you promoted the survey in the first iteration?

Pariser: It was predominately online, blasting this out to our list serves, our networks. We worked a lot through the patient groups that we have with the RDCRN, but also through some of the umbrella groups—National Organization for Rare Disorders, Every Life Foundation, some of the other groups—to try to reach people. We were trying to get as broad as we could, but it was predominately through online awareness.

Barr: With the demographics who responded to the survey, were they all ages or were they primarily 18 and older?

Pariser: They were primarily 18 and older, although we did get some younger people. You could be either the patient yourself or a primary caregiver. It was predominately patients, but it was a little bit more 50/50. We heard from a lot of caregivers. Caregivers would often be parents of a child, for example.

Barr: Did they have very different concerns or experiences than the patients themselves? Some of the parents have other children and other responsibilities. Dealing with the pandemic, sometimes people care more about others than they care about themselves.

Pariser: That's very possible. We don't know yet; we haven't analyzed the data. We should be learning more about that, but that's a very good question. The caregivers also can carry substantial burdens and other members of the family may have to alter their behavior as well during an emergency.

Barr: Have you been involved in any other COVID-related activities either at NIH or outside of NIH, in professional groups?

Pariser: Yes. I was part of the RADx, which is—I forget what is stands for [Rapid Acceleration of Diagnostics], but it's about the diagnosis and testing. They had an underserved population subgroup, so I was part of that for the rare disease considerations.

Barr: What did you do with them? What kind of activity did you do with them?

Pariser: It was trying to stand up these funding announcements and get them out and awarded during the pandemic. It was a real hurry to try to get these announced, advertised, tagged on to existing funding announcements a lot of the times. Then getting them reviewed and awarded—and in a timely manner as possible.

Barr: Oh, my goodness. That's a lot!

Pariser: That was a huge effort. It was led by National Institute on Minority Health [and Health Disparities], who did just an amazing job. A lot of people worked really hard to try to get this out into the testing, development, and into the communities. I did that as part of NIH. I've also been invited, and continue to be invited, to give talks on things exactly like this. What have we done, what have we learned, what are we trying to put out there? We're trying to do that, trying to network to the extent possible. We worked a lot with our investigators, in terms of the delays or the pauses in some of these studies. There have been a lot of extensions and flexibility in keeping the research going—inevitably they were impacted. From the professional standpoint, I'm a physician so I volunteer at a free clinic in the DC area. I've been vaccinating through that and also through the Maryland Medical Reserve Corps doing vaccinating and testing in the community.

Barr: Wow, so a mixture of all things, from writing the grants to actually testing and vaccinating. That must make you feel very good to do the vaccinations; that's what everyone has been saying.

Pariser. Yeah, it's been great. Feeling like you have the opportunity to help—that's always just really nice. We all work in public health; we want to help people—that's why we do this. To have the opportunity to do that was really a privilege. Usually when you're coming after people with needles, they aren't very happy with you. But people were usually just very happy to be there, and just very glad to be getting their vaccines. For a lot of people, this was like freedom. I can go visit my grandchildren; I can go out; I can see my friends; I can see my family. I was just very glad to be a part of it.

Barr: How did you promote the vaccine with those who have very rare diseases? Some are on immunosuppressant drugs so it's still unclear how well the vaccine actually works with them. What kind of conversations have you had with those individuals?

Pariser: We've had a lot of questions on that. My office also runs the Genetics and Rare Diseases Information Center—the GARD program—where people can contact us with individual inquiries. We've had a lot of questions about should they get the vaccine. We can't tell them if they should, or they shouldn't. That's a decision between them and their doctor. We've been relying on the CDC guidelines. About the only contraindication right now is a severe allergy to a vaccine. We encourage people, particularly with underlying conditions, to get vaccinated. The outstanding question now is [whether] people with underlying conditions fully take the vaccine? It looks like people with immune problems, patients with cancer, chemotherapy, underlying immune conditions, certain drugs—it's possible they may not respond as well, but even some antibody has the potential to be protective. Right now, the recommendation is—first of all, talk to your doctor— that most people should be vaccinated. We're hoping potentially the serosurvey might help answer some of those questions as well. Are people being protected, are they generating antibody levels, which antibody levels, which ones are protective, what is the titer? These are all unanswered questions right now.

Barr: Is there anything else that you would like to say as an NIH scientist and clinician but also as a person who is living through the pandemic?

Pariser: Yeah, there's just a lot of people to thank. I guess. In any kind of research but especially something so widespread and so serious and terrible like this. It's just how people, for the most part, have really come together. The scientists, the doctors, the healthcare professionals, the people on the front line, the patients themselves. How communities have just worked together to try to help one another. It's just been extraordinary. Just a big thank you to everyone at every level who has just rolled up their sleeves, and in whatever way possible just really tried to help.

Barr: That's really wonderful. Thank you for all you do. I wish you and everyone continued success and of course health.

Pariser: Thank you and thank you for taking on this project. I think it'll be an important moment in history so I'm looking forward to seeing the finished product.

Barr: Absolutely.