Speaker 1: Ladies and gentlemen, we will begin in two minutes.

Chris Rusconi: Hi everybody, welcome to the plenary research session clinical studies in the ADPKD registry. How you can impact research. I'm Chris Rusconi, the chief research officer of the foundation. In this session I'll provide general information related to clinical trials. Dr Michal Mrug will provide an overview of open clinical trials in the PKD space and Elise Hoover, the Foundation senior director of Research, will provide an update on the foundations ADPKD registry. In the next 10 minutes or so, I'll touch on 3 topics. The first will be a background on what clinical research is. Then we'll talk about what participation in the clinical trial looks like, and then we'll finish up talking about why participation in clinical trials is so important.

So, this slide represents the industry sponsored PKD treatment pipeline. Now, Dr Mrug will dive into the currently enrolling studies on this line talk about that later, but what I want to note here is that it's great to have a pipeline. You might notice that the pipeline is a little bit smaller than what we've seen over the past couple years. As we've seen some late-stage failures, Michal will touch on these in in a little bit more detail, but despite this, one of the things that we're seeing in other research efforts, particularly in the polycystic kidney disease *[unclear] [00:08:22]* consortium, are more companies coming into the ADPKD space in the PKD space in general, and that creates a lot of optimism for us.

We'll see growth in this pipeline over the next several years, and importantly, for this growth to occur as many of you have already done, we're going to need to continue this ongoing collaboration between patients and families with PKD like yourselves and the industry and academic sponsors of these clinicals studies. Now, in addition to industry sponsored studies, there's a number of other academic sponsored studies. These include some that are funded by the foundation that evaluate things like drug repurposing, but also other non-drug interventions for PKD such as diet and lifestyle interventions.

So, now in the next few slides we want to talk about is what clinical research is. So, there's broadly two types of studies that are highlighted here. There's observational studies, these are ones in which researchers analyze health

data, they look for links between the diagnosis and disease progression of symptoms of quality of life, and then there's interventional studies. These are studies that test new ways to prevent, detect, or treat diseases, and it's important to note that an interventional study can be any approach to prevent, detect, or treat diseases that includes drug therapies, but it also includes things like diet or lifestyle interventions and devices.

Now if we talked about clinical trials just the strict definition of what a clinical trial is. These are studies in humans of new treatments or therapies for disease, and they're done to ensure that treatment or intervention is both safe and effective.

Now, one of the reasons we like to share this information with you and really look for collaboration in the population of folks with PKD is that under enrollment is one of the most significant problems facing PKD drug development that takes really two failures. One is trials that just failed to recruit enough volunteers to complete the study. And then the second one is somewhat related, but different, that's fail to recruit patients quickly enough to make study completion feasible. So, again, it's really important that if you're interested that you participate in clinical studies. So that's what clinical trials are, and I think it's worth asking, you know in this vein, so why do some people choose to participate in clinical studies.

So, as we began using the ADPDK registry to help connect patients enrolled in the registry of clinical studies, we've asked about folks' motivation for contributing. And here's what we've learned. First of all, as you can see on this slide, there's a range of motivations that for folks to participate in research. That said, looking at this, the majority of folks participate to contribute to advancing science and treatments and access to specialists and the opportunity to try to innovate treatments are also major reasons that people share that they choose to participate in clinical study.

Now I think probably many of you have heard likely heard this phrase fill in the blank. This is a fill in the blank phase study and so what we want to do on this slide is give a really quick overview of what the purposes of the different phases of clinical studies. So, if you're considering participating,

you understand you know broadly what the purpose is. So, the first phase of clinical studies are phase one study. These are generally small studies. They're designed to gain an early understanding of the safety of a drug, and its appropriate doses and how the drug behaves in healthy people. So, most phase one studies start at least in healthy people.

Now the next phase of clinical testing or what are called phase two studies. This is usually the first time a drug is studied in the patient population, so meaning the group of folks with whom and whom that drug is intended to potentially be a treatment. These studies that say that moderate in size they're designed to gain an understanding of the drug safety in the intended patient population or intended population for use, and the gain early signs of efficacy or is the drug working to really help determine whether the drugs should be studied further and moved into phase three studies.

Now, phase three studies. These are typically large studies they're designed to clearly demonstrate that a drug is effective in treating disease and to further understand the safety of the drug. It's the result of the phase three studies along with that are used by the FDA for review in determining whether a drug should be approved to treat disease, so these are sometimes also called pivotal studies.

And then in some instances there are studies that are called phase four studies. These are conducted after a drug is approved and there really, usually to further demonstrate the drug's efficacy.

So, this is the phase of the clinical studies and now what we want to do is transition and talk about what does it look like to participate in this study. So, the first step in participation is informed consent. This is where you, as a participant, learned from the study personnel exactly what being in the trial will look like. So, you can decide from a position of really truly being informed if you want to participate in this study.

Now these consent forms are really, really crucial, really important to read and they should include the following things listed here. I think importantly, they must include the procedures that you as participant would undergo. They

must include the risk for participation along with any other important details, so it's important to read that informed consent and talk to the study team about it and get all your question answered before deciding to consent or not.

Now the next thing we can think about another important consideration or eligibility criteria for study participation, and these are reasons whether or not you're eligible for a study. These eligibility criteria basically characteristics that must be shared by all study participants, so there's some examples in the green box that are common eligibility criteria like age that your diagnosis, your medical history, current kidney function is, is a consideration for almost all clinical studies as well as current medications.

You know, I think one of the things and we'll talk about this in a little bit. But you know, clinical studies take a lot of time. They take a lot of effort from participants, and so as well as from the study sponsors, and so it's really important that the study be interpretable, and that's really where the eligibility criteria come in. They are there to ensure that the results of the study are due to what is under the control of the study. They really are critical for helping researchers achieve accurate, meaningful results, and importantly, it's important to note asterisk at the bottom of the slide that all study criteria approved by an independent ethics review board. So, they're well vetted and they're really there to ensure that the study is interpretable and also that you're an appropriate candidate for a given study.

And so, what should you expect if you to participate in a clinical trial. So, I think the biggest thing to know, is that your biggest commitment is your time. So, studies require your time for a variety of reasons that can be study visits for checkups that can be study visits for tests such as blood and urine tests and study visits really for any procedure to monitor if the treatment is working and if it's safe and so being able to commit your time is really critical because the best way to get good results is for all participants to fully complete study visits and tests and while just having said that, that participation in full participation is important.

I think what's also really important to know is what's highlighted on the bottom of this slide, and that is participation is voluntary. So, if at any time

you've enrolled in this study, you no longer want to participate you are free and welcome to remove yourself from the study. You can do that by just reaching out to your study team who will help you understand how to leave this study safely. Speaking of safety, let's talk about what are the risks involved in clinical trials.

So, these are obviously really important considerations. Say the risk for each clinical study is different and like we mentioned in the talking about informed consent, researchers are required to tell you all of the possible known risks before you agree to participate in the study. That said, there are some typical risks for participation in studies. First one is just side effects to the medication or treatment being studied. Almost everything will have some side effect. Then they're unwanted events during the trial that can occur that may or may not be related to the study drug. And then finally, there's the failure of the treatment to work.

I think importantly though, is that during the course of the study, the research team will be continuously monitoring your health and safety, whether you're receiving the study drug or whether you're receiving a placebo. Placebo being if you're in a study with a controlled study with the comparator, a placebo is a sugar pill that looks like the study drug. It's so that the study personnel and even you yourself as participant, what won't know what you're receiving. And then finally, when we think about study participation, your privacy and confidentiality are really, really critical. Study sponsors understand that you place great trust in them when you participate and share your data as part of participating. So, all study sponsors take great efforts to protect your identity and data rigorously.

I think there's three key things to consider in that first is that when you consent, you'll be assigned a unique study identification number all your personal identifying information, your name, your contact information your address, your date of birth is separated from your study ID, and that's placed in a secure location. After that, all the data that's collected from you is only identified by your unique ID. We call this deidentified data so if people talk about deidentified data it means your personal information has been

separated from that data. This data is also stored in a secure location, and that's a secure location that's separate from your personal identifiers and then finally when it comes to data analysis, only deidentified data is analyzed and only rarely would a study team connect your name to your data and that would only really be for safety reasons.

If there is a reason that they had to contact you for your own safety or to inform you of safety events in a study, and so I just want to wrap up my portion of the session by sharing this slide that I think highlights the development of treatments that is really a collaboration between researchers and patients' collaborative groups like PKDOC and Clinical study sponsors, so using Tolvaptan in this case. So, in the case of Tolvaptan, many years ago, researchers, including the Foundation's founder, Dr Jared Grantham, developed data in animals that indicated that suppressing vasopressin may be a potential treatment for ADPKD and that really provided sort of what we call that critical start to this journey, the reason to believe.

So hey, this is a reasonable approach and something that we have enough belief in that we're going to take it into studying it in humans. So, after that as you looked across this slide from phase one to phase three, you'll see that is greater than a decade of clinical studies. Not all of them, but some of them are shown here that highlight the incredible commitment of patients. So, I think in that commitment is in terms of both numbers of participants as well as a time requirement for studies. Some that are short, 5 to 8 weeks, but some that are very long. Multiple years of commitment for participation and I think that really highlights the role of you all as patients and fueling and facilitating the development of treatments.

I think the last really take away message here is that you're truly essential to development of all treatments for PKD and so with that, what I'd like to do is hand the session over to Doctor Michael Rug, who, among other hats, chairs the scientific advisory panel of the Foundation. Welcome, Michael.

Michael Mrug: I would like to thank Doctor Rusconi for a nice introduction and for a very nice introduction of the important concept of the PKD treatment pipeline that

stands from the discovery through the phase one to three the track approval and post marketing studies. Can I get next slide?

So, I'm not going to talk about the individual components of this pipeline that were covered by Dr Rusconi. Instead, I would like to focus on the studies that are recruiting or will be recruiting in the near future. And but before going there, next slide, I would want to mention studies that were not successful and point to the fact that the current successes they rest on the shoulders of studies that have failed. And this is these are the examples of terminated studies. The studies that were stopped early and will not start again, it included the studies of Sirolimus and Everolimus, Triptolide Venglustat or Lixivaptan. And these studies are failed and were terminated either due to lack of efficacy or there were issues with safety or a high drop out.

I would like to focus or mention the alert and action studies that explored in phase three safety and effectiveness of Lixivaptan. And this study was recently discontinued due to safety concerns because liver toxicity differed little from therapy already on the market. So, thank you all who participated in this study and all these studies that didn't help us to move forward directly and were terminated.

So, what are the studies from the PKD treatment pipeline that are currently recruiting, and I'll try to provide a quick overview and then discuss these studies individually. So, one of them is the Pravastatin study in early ADPKD that is done by University of Colorado. The other Bardoxolone methyl that is done by many sides in the United States, then the regulars are compound 8429 that is targeting micro-RNA and then the behavioral or diet studies that are done at University of Colorado, in Australia.

So, let me discuss them specifically. So, the Pravastatin, Pravastatin study is a repurposing study of effectiveness of Pravastatin, which is an FDA approved track for hypercholesterolemia. The study involves an oral drug Pravastatin use versus placebo on every day for two years. The studies looking for 200 participants who have ADPKD with total kidney volume over 500 milliliters of eGFR over 60. Well controlled blood pressure and no recent use of Tolvaptan, fibrates, niacin or cyclosporine.

The next compound that is being explored currently as RGLS8429, which is one of these compounds that target micro-RNAs. And it's a new compound that replaces recently studied RGLS4326. It's injected subcutaneously and this is a phase one study of safety tolerability. And they are looking for 32 participants for this study and the participants should be 18 to 55 years of age with ADPKD diagnosis, eGFR more than 90 and BMI 18 to 35 and no current use of Tolvaptan.

The next compound investigated is Bardoxolone methyl. They studied in a Falcon study, which is a phase three study of safety and effectiveness of Bardoxolone methyl. It uses this oral drug versus placebo. The drug is taken every day and the study is going to last two years. The study is looking for 850 participants. 18 to 70 years old with diagnosis of ADPKD, eGFR 30 to 90. Well controlled blood pressure and no current use of Tolvaptan.

Among the behavioral modification or diet modification studies, I would like to highlight the study of caloric restriction that is done by University of Colorado in Denver. It's a two-year group based behavioral weight loss intervention that is based on 30% reduction in caloric intake and increased physical activity. They're looking for 126 participants, 18 to 65 years old with diagnosis of ADPKD and increased body mass index 25 to 45. The renal function eGFR over 30. Height adjusted total kidney volume over 16 and no current use of Tolvaptan.

So, I would like to also mention studies that are not yet recruiting but will likely start recruitment in near future. That includes the study of Tolvaptan in ARPKD. And the studies of Metformin in ADPKD, Hydrochlorothiazide in ADPKD. Since it's not much known yet about these studies I want to point you to an excellent website, a resource provided by NIH called ClinicalTrials.gov, that allow you to explore over 400,000 studies in most countries and you can basically explore this database by typing the condition here in this case, for example, polycystic kidney disease and then looking at a subset of studies and marking, looking at the studies that are recruiting or not yet recruiting.

So, we don't need to look at studies that were on already completed, but only those that are looking for participants for future or potential participants. And if you do that, what you will get on the next page, and this is just an example of the two top, of the next of what you may see, you know the studies that are recruiting and are the studies that are not yet recruiting and details are provided in terms of what is the title of the study, so you may get some insight about what is the study and the location of the studies. And often for the studies that are not yet recruiting the information are doesn't include location or some other details may be missing. So, but you can click on the individual names of the studies and learn more about these trials.

Now it's maybe sometimes little confusing to navigate this website and there is a lot of information provided once you start to look at the details of individual trials. So, to make it easier for patients PKD patients to navigate which clinical trials they may be able to participate in. Our PKD foundation has established the clinical studies tool. That is an outstanding tool to navigate this field of clinical trials in ADPKD research and Elise Hoover is going to cover this in greater detail in the following talk.

I would like to also want to mention that while most of the studies have been historically focused on autosomal dominant polycystic kidney disease, now there has been a great progress in our research of autosomal recessive polycystic kidney disease or ARPKD. Looking at both the biomarkers of the kidney and the liver disease are establishing the databases but also the first treatment trial using Tolvaptan which is not yet recruiting.

There also multiple other studies that are going on that I didn't mention that are looking at the Pathobiology of ADPKD or ARPKD and the other molecular pathways that may be important for the disease pathogenesis. Including our repositories and registries and one of them, the PKD Foundation ADPKD registry is a focus of the next talk by Elise Hoover.

Elise Hoover: Excellent, thank you Doctor Mrug. Hi everyone, so now that we've heard about the clinical trials in the pipeline for PKD, we'd like to share reviews and details of the foundations own research project, so we'll show what it looks like, how we try to return value to participants, and then some tools

we've created to help make research more accessible. So, why did the foundation decide to build a registry? So, this is a collection of individuals with a given disease who provide data on their experience and went late to do is talk about how it compares to a clinical study, which is a classic research design that hopefully you're familiar with, especially after Dr. Rusconi talk.

So, in a clinical trial, you are limited to sign up based on inclusion criteria, but Dr. Mrug pointed out in many of the ADPKD studies. However, in a registry there are few limits to age or disease stage. In fact, in ours anyone with the disease is invited to join in a trial, there is a maximum number of participants and ultimately the study will close for enrollment, but in a registry, if we can help it, the study never closes, and participants continue to sign up as long as there's interest. The kinds of study, the kinds of data you can collect in a clinical study are pre-selected based on the protocol, but in a registry, we have more flexibility to add new questions and year over year, repeat them. Ask them in a different way which is really exciting.

One important similarity, though of course, is this idea of confidentiality. Dr Rusconi walked us through what that looks like and both clinical trials and registries will separate data from identifying factors like your name, and I'll talk a little more about why this is a little different in the registry later. Ok, there are also different kinds of registries, so traditionally clinic-based registries pull data from your electronic health record and the only opportunity you have to engage with the researchers is when you sign that consent form. The data itself also largely focuses on tests and procedures from the clinic.

However, in a patient powered registry like ours we use they are called PROs or Patient Reported Outcomes and this data is provided directly by patients and because we're doing that, we rely really heavily on engagement with the registry. So, we ask people to continue to log in and come back and answer more questions. We can also collect data that's very unique, such as quality of life, pain, fatigue that is really hard to get from the electronic health record.

So, in the foundations history we have committed to funding and supporting research, and this is the first research project that we have managed ourselves.

We're really excited about it. It was launched almost three years ago on PKD Awareness Day. It's hosted online and we hope that the goals of the program will go far to help advance research to find better therapies and cures utilizing the patient invoice

Alright, so before we get into some of the really neat data from the registry, I just want to go ahead and repeat this slide in terms of the registry. You know we saw this from Chris earlier. This is how we protect participant privacy and confidentiality in the registry. So, because we are online patients access the registry using a secure web-based portal. The portal then automatically assigns the ID to your data, so there's no need for a human to be involved at all in this. There is no steady team. There's no doctor. The system does this for us. And then of course those details are stored in a separate location that is secure and then as you fill out the modules and the surveys, all of that data is assigned to our unique ID, similar to a clinical trial. And then what we really love about our program is that we have advisory groups made-up of researchers, clinicians and patients, stakeholders, and so they help us decide how to use that data. And we're also building a data sharing portal related to this year for researchers.

Ok, so now the good stuff. This is the data from our 2021 annual report. We intend to create one of these every year to show you the impact of the data we're collecting. As of this week, over 2600 individuals have signed up to participate with us. They are from all over the United States. They have many different stages of disease. You know, as you can see though, there is a need to increase both racial and gender diversity in the program, and that's one of the foundations goals for the next year.

Here's an example of how we use the data, so these are results from our family history module. And so, genetics of ADPKD tells us that an affected parent has a 50% chance of passing that gene on to each child, but we want to know is what do these families actually look like. So, what we did is we broke it down by number of siblings. And if you look here, let's say someone who has two siblings, 45% of those who had two siblings, they were the only person in their family with the disease if other siblings with the disease. And then

20% all three siblings had the disease so. It's just really interesting to see how that falls out. We hope that you'll look and follow the path for your own situation and see how those numbers compare.

Ok, and we also have a really great tool for helping to recruit for studies in the registry, so our core questionnaire ask details about disease that would serve as those inclusion criteria for a study. So, kidney function, age, symptoms. The system will then automatically create cohorts for us or groups of patients that might qualify for that study. Again, we don't know those names, we just know that we found 250 people who may qualify for a study, and we asked the portal to send you an e-mail that tells you more about how to participate. And what's important for us is that we leave the power in that individual hand to decide if they want to participate or not in that study or reach out to the study team. We just want to make sure that we spread awareness of the research that is ongoing.

And here's another example of how we use the data. So, this is a poster that our research team presented at the National Kidney Foundation annual meeting this past April. So, this is where we're really starting to reveal insights into how pain impacts those with ADPKD. We used a validated pain scale. We asked about short pain, dull pain and fullness or discomfort, and then we broke that data down by those who were pre or post-transplant and at different stages of disease. And what we learned is that while we do see some differences in impact of pain and on quality of life based on what stage of disease someone might be in, pain has an impact on many, many PKD patients across the spectrum. So, we believe that knowing more about kidney pain will help us to inform clinicians about the symptoms that impact their patients' daily lives.

Ok, it is also important to our team that we continue to find ways to show participants how we're using their data and what we're learning. So, this here is our dashboard. So, if you are a participant, you would log in to your platform and you would see the dashboard and get an idea of some of the data we're learning and then where you fall in there. We also have a quarterly newsletter, so this highlights a few charts. It talks about why that data matters.

It shows a preview of what's coming next and then we also share some goals for the quarter with the participants. And then, as I mentioned before, we have an annual report every year. So, not only do we try to present some of the data in this report, but we pull in participant testimonials or narratives that help us match those tables and figures to a real individual's life and the impact of disease is having on them and their family. So, we try to do a mix to really help us all understand what we're seeing in this data.

Ok, so for those of you who may not already be signed up, I hope you consider it. This is I'm going to walk you through how that process works, so this is our home page pkdcure.org/registry. And you go here, either sign in or sign up. If you go to sign in there is a place to direct you to sign up, but this is where you would log in if you're already a participant. So, we ask you for your name, your e-mail and a password. We asked a little bit about you, your gender, your date of birth. We then ask you for your phone or address. These are optional, but at the least we'd like to know what city and state you live in. And then this is the consent form. So, as Chris mentioned, a lot of really important information in this form, and there's a lot to scroll through. So, what I recommend you do is you click at the top here, or you can just download a PDF and take your time and read it and ask questions before you come in here and check these boxes and agree to participate.

All right, in addition to the registry, another one of our recruitment tools is our act alerts, so this is an e-mail listserv that you can opt into. We have one for auto symbol dominant PKD and autosomal recessive PKD and whenever we hear of new studies that are enrolling, we will try to send an e-mail to let you know. Sometimes we will do it based on where the study sites are located in the US. Sometimes we'll just send it to everyone to let them know.

And then, as Dr. Mrug mentioned, we have our clinical studies tool. As fantastic as ClinicalTrials.gov is, it can be a little hard to sort through all that information. So, we've done our best to help filter that down for you and share with you what participation looks like in a clear way. So, you go to find a study which is at the top of that page there. You have the option to fill out some details to help you sort through the studies. What is your diagnosis?

What is your latest kidney function? How old are you? And then we'll pull up a list of studies that you believe you may qualify for. We also have an option to sort by location, so there's a lot of studies on there right now. We do our best to keep it updated and please check it out and let us know what you think.

Ok, so that's it. Thank you so much, you know I can't say enough how important it is that you know patients like you sign up for research to participate for the registry for clinical studies. We wouldn't have we wouldn't know half of the things we know now about the disease without those who signed up to participate. So, thank you so much and hopefully we can answer some questions now. Alright, let's start for one for Dr. Mrug, So, look around you or clinician you see patients when you are encouraging someone to sign up for a study, what's one of the most important things that you tell them?

Michal Mrug: I think it's you know a lot of times the study when you learn about it seems exciting, and I think that what is important to recognize that and all these studies, these are experiments, right? So, there often are side effects, and I think that sometimes people make it blinded by that hope you know that this maybe it. And not to take into consideration appropriately, the potential adverse events that may happen. So, I think that you know being aware that you know this may be grateful but being aware that you know these drugs could potentially hurt you and weigh for it you know early and trying to make the decision whether to participate or not I think it's important.

The other thing I think that is important, and Chris mentioned that is the time commitment you know, one needs to realize that it's not you know if you subscribe for a study, it's not just, you know you'll do it and you know a couple of weeks later, you know perhaps you may withdraw if you can do it and always you can do it. But it's just a waste of time of the study team. It's the waste of resources. And its waste off your time, you know as a participant. So, I think that are taking into consideration all those things that might be important, including the time commitment, because sometimes people don't realize how much time it may take is important before making the final decision whether to participate or not.

Elise Hoover: And Chris here's one for you. Why is it so important that participants privacy and confidentiality is protected? Why does that data needs to be secure why are we all trying so hard to do that?

Chris Rusconi: I hope the answer to that question is somewhat self-evident, right. I mean obviously I think there's a lot of reasons there. I think the first one is that when you're contributing to these studies, these are experiments. There may be learning things that haven't been learned before. You may not know condition and it's important that data be private so that it's really only used for its research purposes shared back to you if it's important for your health conditions, but that it's not available for to your insurance company. It's not available to somebody trying to sell something, it's just really critical that information be maintained and it's in privacy and confidentiality because it's only intended use is for its research purpose. And I think we all know, like any of us, if we as we participate in clinical studies from time to time that violate the trust of the participant it's very unlikely that people will participate in clinical trials, and so it's just really critical to the entire endeavor to maintain that trust relationship.

Elise Hoover: I see a few questions here that I can answer also, so we had someone ask where they can get the annual report from the registry so that is available on our website pkdcure.org/registry. Or if you go to the PKD research exhibits in our PKDCON platform it is linked there as well.

And then there's also a question here about merit reviewers. So, for anyone who is familiar with this, merit review, are many organizations will use a stakeholder to help us assess the potential impact of research grant applications. And we at the foundation have a group that is made-up of both researchers and stakeholders. It's called the stakeholder reviewer panel and we open up applications for that panel every October, so that will be coming out soon and I believe the website is pkdcure.org/stakeholder panel reviewer but feel free to message me directly on the platform and I can also send you some information.

Just checking out another question here. Ok, Dr. Mrug, we have someone who is very astute and noticed that the Bardoxolone methyl falcon clinical

study used to have a smaller number of participants they wanted to enroll and they upped it to 850. Could you tell us why that might happen?

Michal Mrug: I don't know this you know this is an internal decision you know of the person that run the study.

Elise Hoover: Well, we'll do our best to help them recruit. Ok, any other questions let's see. We have what percent of the 2500 enrolled in the registry RPKD patients. All of them 100%. We ask that anyone who has a diagnosis of a ADPKD or suspects that they have the disease to please sign up with us. The core questionnaire ask that question do you have an official diagnosis and if you don't we ask you some questions about why you think you might have ADPKD. Is it symptom? Is it family history? So, those are questions that we're really interested to know, and we hope that you consider signing up. Ok, I think that's all the questions we have. Any final thoughts Chris before we sign up?

Chris Rusconi: Well, I'll make one. I think it relates to the comment that Dr. Mrug made about time and trial participation and that is one of the one I think the really exciting trends we're seeing in a lot of clinical trials is much more engagement with the patients and participants who might be involved or considered being involved in the trial in the design phase to really understand is this trial design something feasible for the patient population in which the treatment will be studied and so I just again encourage you if you have you ever had the opportunity to weigh in or be involved in a panel to get feedback about the design of a trial that you consider that, because again, you know, as we've, I think we've highlighted time is one of the really critical factors for both the participants in the study sponsors and this trend of going and actually talking to the people who would be involved in this study to understand what design might work for them and their family, or what designs won't work is, I think, a really important trend and I hope we can all support that and that all I think also will make it at least lower the barrier to participation in trials and make it a better experience. Just the last two cents on that.

Elise Hoover: Absolutely. And the foundation does have a volunteer opportunity called our Community Reaction panel and one of the goals for that group is to put ideas

about protocol design for studies in front of them and get thoughts. So, if you're interested in giving some feedback and getting your voice into the design of clinical studies. Please go do check that out and consider volunteering for that role. All right Dr Mrug any final thoughts?

Michal Mrug: Well, I would like to say that from my view you know like ADPKD Foundation doing outstanding job in trying to educate our patients, caregivers, which I think is very important because the information empowers ones you know to make the right decisions. You know we cannot have physician to decide for you, so learning and be able to, you know, be empowered to make the best decisions for yourself. You know that I think is very important. And the foundation here is a catalyst you know to get that information to tried hands.

So, I would like to thank you know what you and Chris, what you are doing and the foundation because I think it's outstanding. And then I would like to encourage patients and our families of PKD patients to try to seek that information and then use it and use it to your own benefit using your common sense. And thank you everybody who participated in clinical studies. Thank you.

Elise Hoover: Yeah, and I'll close this out just by saying that there is one more session where you'll see Chris Rusconi speak today. It's the last session of the day, and he's going to tell us about our general research program, and we have one new program launching in the fall that he'll be speaking about. So, if you have time and you can stay with us, highly recommend attending that. Well, thank you so much everyone. If you have any questions, please go ahead and connect with me on the platform. Our research teams e-mail is research@pkdcure.org. Happy to answer any questions.

[Audio End] [00:51:04]