IPWSO Leadership ECHO challenge November 30, 2021: Improving clinical trial participation

We were pleased to hold another fantastic Leadership ECHO session on November 30, 2021. In this session our Challenge was presented by Diane Webster, Research Director, PWRFA, who helped us to consider how we might accelerate the development of new drugs for PWS by improving enrolment in clinical trials. We discussed the barriers to trial participation, expanding our recruitment pool, what's working well, and the importance of finding the right trial.

Diane highlighted a fairly well-established pathway from basic science to the clinic, the length of time that that can take, the different phases of clinical trials and how you start with a small number building up to perhaps 150 in later trials.

In the field of PWS we need a successful trial that produces some new treatment that parents and people with PWS can say to themselves, "Yes, this is making a difference." and we haven't got that yet.

In the discussion that followed Diane's talk, we identified that there is a lot of enthusiasm for participation, but the challenges with practicalities around research must be considered carefully.

Diane Webster: Improving clinical trial participation

The video link is above. You can read a transcript of the Q&A with Diane which followed the discussion below, The PDF is available here.

Diane also referenced a previous talk by Nathalie Kayadjanian on *"Clinical Trials for PWS"* as an excellent resource if you would like to learn more about the topic.

Please note this document is abridged from audio transcription of the Zoom session. Some errors resulting from the transcription process may be present.

Hosts: James O'Brien (JOB) and Tony Holland (TH)

Comment: I think it's a question of money, because I think most families are interested, but it takes time to travel with the child, not to go to work those days, for example, and there's so many things as well. The study that we had with adults with PWS, there was paid staff who travelled with the adults with PWS to the research

centre. It takes so much time from the families and if they have brothers and sisters and so on. So it's really some practical things more than the willingness to participate.

DW: I like that idea. We often get told that the costs will be reimbursed, but the idea of reimbursing a companion to support the travel is a lovely idea.

Can I ask, the remote or telehealth and/or similar site visits - How appealing is it to have a research nurse come into your home or to be able to participate remotely over telehealth?

Comment: I think it's a perfect idea that someone comes to the home. Instead of that you should travel and go into rooms where you have not been before and, what's going to happen and when will I have my next meal and all these concerns. It's much better if a nurse comes to the place where the person with PWS lives, it's a perfect idea. But there's a problem, for example, if you have to do a sleep study or something, then you come to the place, but for collecting blood samples and giving instructions and interviews, it's much better to come to the home.

JOB: And you've mentioned Diane, telehealth or doing it over a distance. I think it might narrow down the number of trials, but any other thoughts from the floor regarding whether trials can be done over distance with telehealth?

TH: I think the answer to that is yes. I mean obviously there is an issue around safety and things like side effects, but if we just for the moment put that aside, I think a lot of the assessments that are done in clinical trials can be perfectly done using Zoom or whatever and certainly in the trials that I've been involved in here over COVID time we've done more and more through Zoom, and I think as long as there's still a good contact with the family, I think it's been acceptable.

DW: So the contact as in having one person you can pick up the phone to who's the study coordinator or something like that. Just someone you know you can get rather than a switchboard. Is that what you mean by a known contact?

TH: Very much so I think that's really important, and we had an example recently where one of the participants in the destiny trial had some concerns and they knew exactly what to do and they were able to get the person running that trial. And I think that was fine. They're also still now coming to see us, but not on every occasion. And I think the other thing to say is we don't think about technology enough. In a different context we're using Fitbit to measure outcomes and increasingly things like Fitbit can be used for sleep assessments, the newer versions of Fitbit and so on.

DW: There's some really interesting potential in that in the wearable monitoring, wearable technology space, definitely.

Comment: If the researchers can't go to the home of the clients, I think it's really important to provide an area where the clients do come in that is easily accessible with

parking, because I know you know if it's in a major hospital it's a nightmare to go in there. If you're in there just for half an hour and you have to do that every 2nd week, so providing an accessible research room or centre I think is important.

DW: That makes a lot of sense, especially, if I find it stressful navigating on and off trams, which route, which room, which part of the hospital? So really clear instructions, accessible and simple.

Comment: Yes, and always be aware of this tendency for hyperphagia. You should not have a machine serving hot chocolate in the room where the person with Prader-Willi will come, because then you can have a tantrum. There's so many small things, and the all the meals should be planned so that food security is kept. It is so important and sometimes researchers who are not so often in contact with the person with PWS do not think about it. I think it's so important. I have many examples where it didn't work.

Q: I was actually going to talk about and ask Diane about the challenge that we have in regards to the reach, and how we reach potential PWS families to participate. I know just speaking from our Australia point of view it's an increasing challenge to have our registries so that we can share them and have a collaboration between the Research Foundation and the Association. I imagine that would be the same in every country, would I be right?

DW: I think with good communication we can always do better. Certainly the registry landscape in Australia is complex.

Comment: I think we are talking about a lot about the processes just now, and is there any way that we can have standardised processes of our research technique or whatever, and make that known to everybody. So that we can carry out similar or the same process in every country. That is my suggestion.

DW: So, are you talking about at the level of say, food security, or these are the things that would make a trial work for someone with PWS and their families? Or are you talking more at the level of study protocols and ethics approvals, and that kind of thing?

Comment: I think it's more on the families, for those who participate. I'm talking about the participants of the clinical trials.

DW: The notes I'm taking today I will be forwarding on to a couple of the companies that we were working with that this would be our suggestion for how you can get good engagement. It's one of those situations where it benefits everyone. If we can set up an environment, so yes, some protocols that work that have been really deliberate in addressing the needs of the people who will be participating in the trial, then the data that they get out of those trials will be presumably better and more robust as well, so I think that's an excellent suggestion.

And thank you for that question.

JOB: You mentioned Australia, US, Europe. Is there a real barrier for drug companies, in particular the big major American drug companies, to trial in Asian countries?

DW: I actually don't know. I know there are some language barriers around some of the endpoint measures where for example, the HYPERPHAGIA questionnaire - I believe you need to have very strong English to be able to answer that one, but I don't know in general. That's not something I can speak to, I'm sorry. It would be an interesting one to know, because we lose so much if we don't look globally. If we only look to English speaking countries, then we're missing out.

JOB: Thank you so much, Diane, really a stimulating set of questions you had for us and thank you for your summary of how things are going for the Prader Willi Research Foundation Australia and keep up the good work there.

DW: Thank you.

Thank you very much to everyone who attended the session and participated. We look forward to seeing you again at the next session on the 11th of January.

Ends.