Tips for Conducting a Successful Clinical Trial In Prader-Willi Syndrome

PWS Clinical Trial Consortium

A PUBLICATION BY THE PWS CLINICAL TRIALS CONSORTIUM

WWW.PWSCTC.ORG

TIPS FOR CLINICAL STUDY SITES

BE IN CLOSE COMMUNICATION WITH THE CAREGIVER FROM THE START.

People with PWS have a challenging behavioral profile and caregivers are under intense pressure to manage their person with PWS. You can help them through the clinical trial process by initiating communication early.

NOTE: Some caregivers/patients are quite research-savvy, and some are new to research; be ready to tailor your communication accordingly

BEFORE THE FIRST IN-PERSON PATIENT VISIT:

Conduct an in-depth virtual prescreening to determine eligibility. This ensures that participants traveling to the trial site are most likely eligible for the study.

A brief phone **call with the caregiver** ahead of the study visit should include:

- Discussion of behavior triggers, e.g., topics to avoid discussing in front of the person with PWS
- Review of the visit agenda, covering what to expect during the study site visit (e.g., where to park, map of site area, flow of visit, expected duration, food options & any planned meal breaks)
- If there will be a blood draw, remind the caregiver that the person with PWS should drink extra fluids the evening before and again 1 hour before they arrive for the visit.

Beta test any patient-facing data capture apps, diaries, and technology so you are prepared to demonstrate these items during the patient visit

TIPS FOR CLINICAL STUDY SITES

Tip #3 Steps for a Successful In-Person Visit

- Take time at the start of the visit to get to know the person with PWS. If the person with PWS is not one of your regular patients, develop rapport.
- There should be no food visible at the site. Avoid having the person pass near cafeterias or other food sources if possible.
- If fasting labs are needed, start early in the day & complete pre-eating tasks as quickly as possible.
- Individuals with PWS have challenges transitioning - allow time and give prompts to facilitate smooth transitions between procedures. A visual schedule may be helpful.
- Caregiver questionnaires and data gathering of sensitive information about their loved one with PWS should be done without the person in the room. A second staff person may be needed to take the person with PWS during those times so the caregiver knows that the person with PWS is occupied and they can better focus on completing the assessments.
- Take cues from the caregiver regarding discussion of person's behaviors in front of them (e.g., skin picking, temper outbursts, food can trigger emotional outbursts or difficult behaviors).
- Flexibility is key (e.g., urine sample collection, order of trial activities) - note that the person with PWS may refuse some assessments, so prioritize critical assessments and be willing to compromise if necessary and allowed within the protocol.
- Train the staff nurses to help hydrate and warm the arm of the person with PWS prior to any blood draw to maximize their chances of getting a blood sample. Vein finders can be helpful with this population.

TIPS FOR STUDY SPONSORS

Limit the amount of travel required

Travel with individuals with PWS can be very challenging. Design study protocols that limit the number of in-person visits as much as possible.

- Consider remote options for lab studies– e.g., LabCorp or local lab
- Pay study sites for prescreen time so that potential participants can be screened remotely, ahead of the 1st visit. This will eliminate participants who will not be eligible before they travel to the trial site.
- Consider virtual follow-up visits and employing wearable technology, as appropriate and tolerated by the person with PWS. Note: Fabric/Nylon wristbands are better tolerated than a silicone band.

Limit the length of the study visit

PWS behaviors can be challenging and prolonged study visits can lead to increased behavior outbursts and anxiety. Consolidate visit tasks as much as possible to keep the visit length as short as possible.

Collaborate with clinician researchers and families on protocol development

Early collaboration with knowledgable clinical sites and experienced families will ensure feasibility and minimize burden.