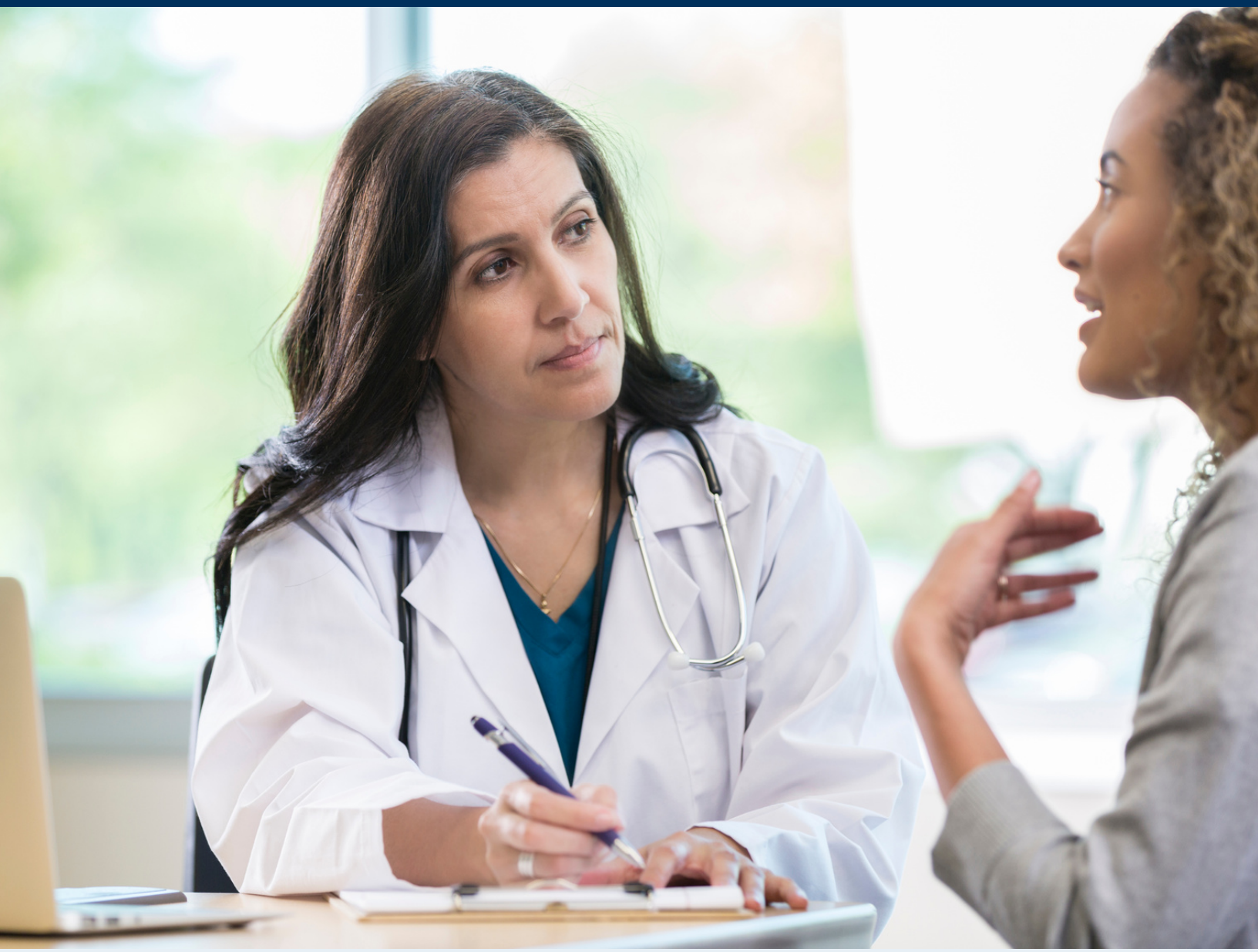

Improving the PWS Clinical Trial Experience

RECOMMENDATIONS FROM PRINCIPAL INVESTIGATORS AND STUDY SITE COORDINATORS

PWS Clinical Trial Consortium

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Introduction: This project was conducted by the PWS Clinical Trials Consortium to seek feedback from PWS clinical trial sites regarding how to improve the PWS clinical trial experience. Through this process, several recommendations were shared to improve clinical trials and to help better ensure their quality and completion. This publication summarizes interviews with clinical trial site principal investigators (PIs) and research study coordinators (RSCs), and their recommendations to improve clinical trials for PWS families.

Methods: Hour-long semi-structured interviews were conducted with 7 PIs and RSCs from several PWS clinical trial sites across the US and 1 in Canada. All those interviewed were working in academic medical centers. Those that participated in this study had a range of experience conducting PWS clinical trials with all working in the area of PWS for at least 9 years. PWS clinical trials interviewees had worked on 3 to 7 previous PWS trials. All interview participants had also conducted clinical trials in other diseases and syndromes.

Interviews were conducted with 7 principal investigators and research nurses who have participated in 3 or more PWS clinical trials.

During the interviews PIs and RSCs were asked to discuss their experiences conducting PWS clinical trials including what has gone well, what aspects would make them want to conduct additional clinical trials in PWS, and what aspects have been most challenging. These interviews have been analyzed to help us better understand the factors that increase the likelihood of trial participation and completion, as well as bottlenecks and challenges.

2 primary factors determine if a PI will participate as a trial site.

1: they “believe” in the drug and are excited to share it with the community

2: the protocol is “feasible and realistic”

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Results: PI's and RSC's shared several aspects that increased their satisfaction working on the trial and we well as their productivity. Many interviewees stated that the way pharmaceutical companies worked with them and their team was crucial to their success and flexibility and timely communication were frequently mentioned as important contributors.

FACTORS THAT LED TO SUCCESS IN CONDUCTING & COMPLETING THE TRIAL

- Being approached early in the process and being open to feedback on the protocol from the PI and their team helped ensure the feasibility of the trial
- Feeling like a “collaborator” with the company, not an employee
- Feeling part of the “team” with the company and other trial sites
- Being able to obtain upfront costs to get the trial started and cover protected time for the PI; especially getting support for ‘start-up’ costs

FACTORS THAT ASSISTED IN THE RECRUITMENT OF STUDY PARTICIPANTS

- Advocacy and recruitment support from patient organizations (FPWR, PWSA, IPWSO)
- Being able to build into the budget extra time for recruitment and “hand-holding” for families to get them into and thru the trial
- Coverage for travel costs for families and ease of access of this for families, e.g., expenses covered up front, fast reimbursement for costs

FACTORS THAT MADE TRIAL PARTICIPATION AND COMPLETION MORE DIFFICULT

- Sponsor’s and/or CRO's limited knowledge of PWS
- Sponsor's reluctance to work with PWS experts
- Sponsor's reluctance to incorporate feedback from PIs
- Unrealistic timelines & time commitments for conducting the trial
- Burdensome data & safety reporting requirements; “too many assessments”
- Trial finance/budget challenges (e.g., not enough budget built-in for recruitment)
- CROs being “pushy” with trial sites before the sponsor has all the documents and other aspects ready to go. “A lot of hurry up and wait.”

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Top 10 Suggestions for Improved PWS Clinical Trials

1. Include input from patients, patient advocacy groups, and experienced PWS investigators in the protocol at an early stage.
2. Reduce questionnaire burden by identifying priority assessments that are required to be completed.
3. Utilize remote visits for follow ups whenever possible to ease burden on families.
4. Reduce burden on sites and families by limiting the participation time commitments, the number of office visits required, and the number of procedures required per visit.
5. Ensure patients and families are fully and easily supported with travel and logistics.
6. Ensure sites are fully funded to conduct the study. Consider that PWS trials can require extra time for enrollment.
7. Communicate frequently with trial sites and families. Ensure that trial sites have knowledge abut any changes before families do.
8. Consider adding qualitative interviews and other measures to address the challenge of obtaining accurate hyperphagia data.
9. Provide extra support to junior faculty who are running trials. Offer opportunities for publishing, and presenting data.
10. Have a “clinical site problem solver” who can work across sites. This should be someone with expertise working with people with PWS and experience running clinical trials.