



Improving the PWS Clinical Trial Experience

RESULTS FROM A **CAREGIVER SURVEY** OF PWS CLINICAL
TRIAL PARTICIPANT EXPERIENCES



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Introduction: This project was conducted by the PWS Clinical Trials Consortium in 2022 to seek feedback from PWS family participants in clinical trials regarding how to improve the clinical trial experience for participants with PWS and their caregivers.

Methods: As part of a study exploring clinical trial experiences in PWS, a survey was developed for families who have participated in at least 1 PWS clinical trial. The goal of the survey was 1) to identify factors that have the greatest influence on clinical trial participation; 2) to identify factors that make it easier for families to participate in trials and 3) to identify challenges that made participating in trials more difficult.

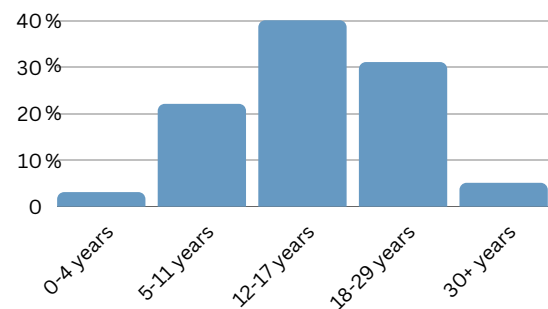
Background: 74 caregivers completed a survey sharing their experience of participating in 1 or more PWS clinical trials. At the time of trial participation, the person with PWS ranged in age from infants to over 30 years, with the largest number of participants in this survey in the teens and young adults age range. 52% of those with PWS whose caregivers participated in the survey were female and 48% were male.

Nearly 40% of those surveyed have participated in multiple trials.

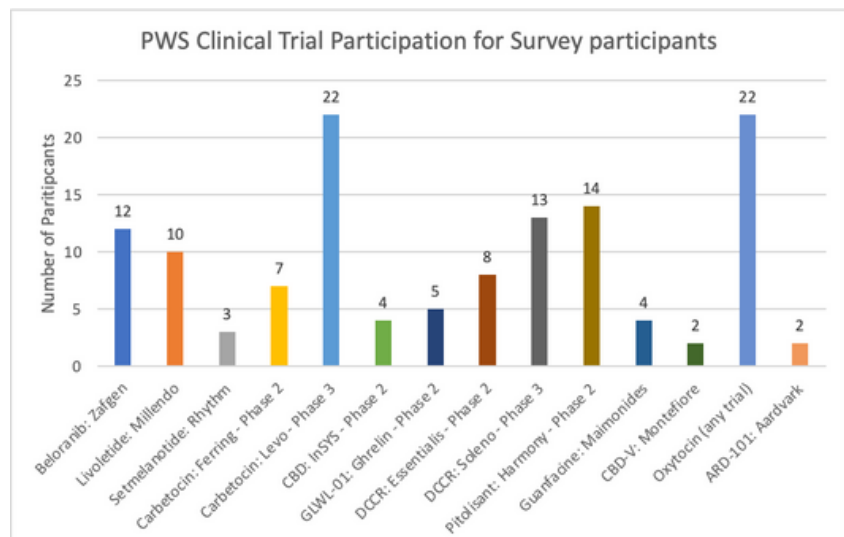


74 caregivers participated in the survey. Of those surveyed, nearly 40% had participated in multiple trials.

Current Age of the Person with PWS



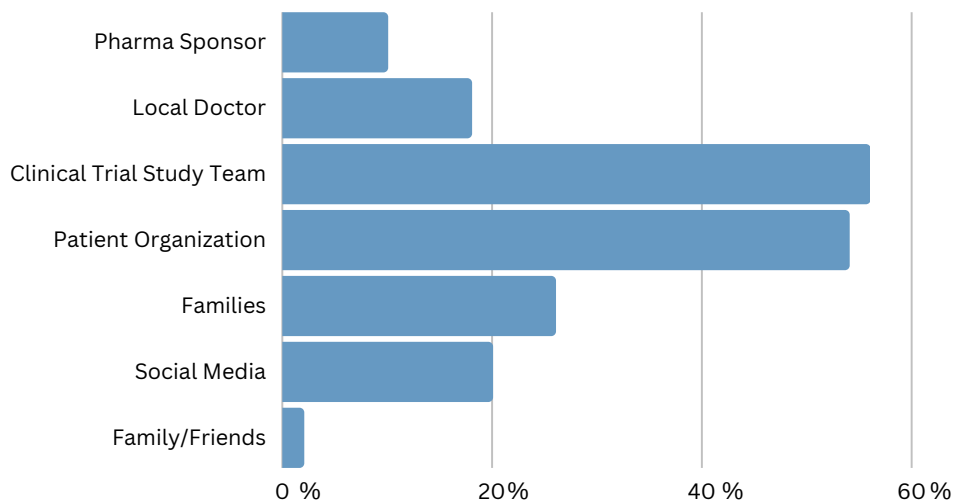
Those who participated in the survey were able to reflect on their experiences from a wide range of trials conducted in the PWS space over the last 10 years. Note some had participated in more than 1 of the trials listed.



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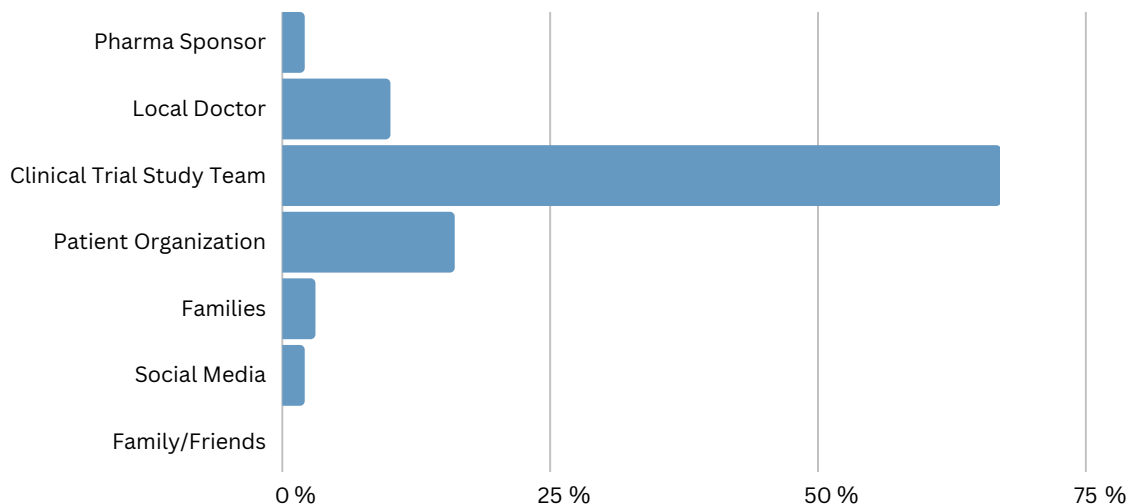
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Where did you get information about the trials you participated in?



Caregivers tend to rely mostly on the clinical trial study team doctors and PWS patient organizations to get information about trials that they might participate in.

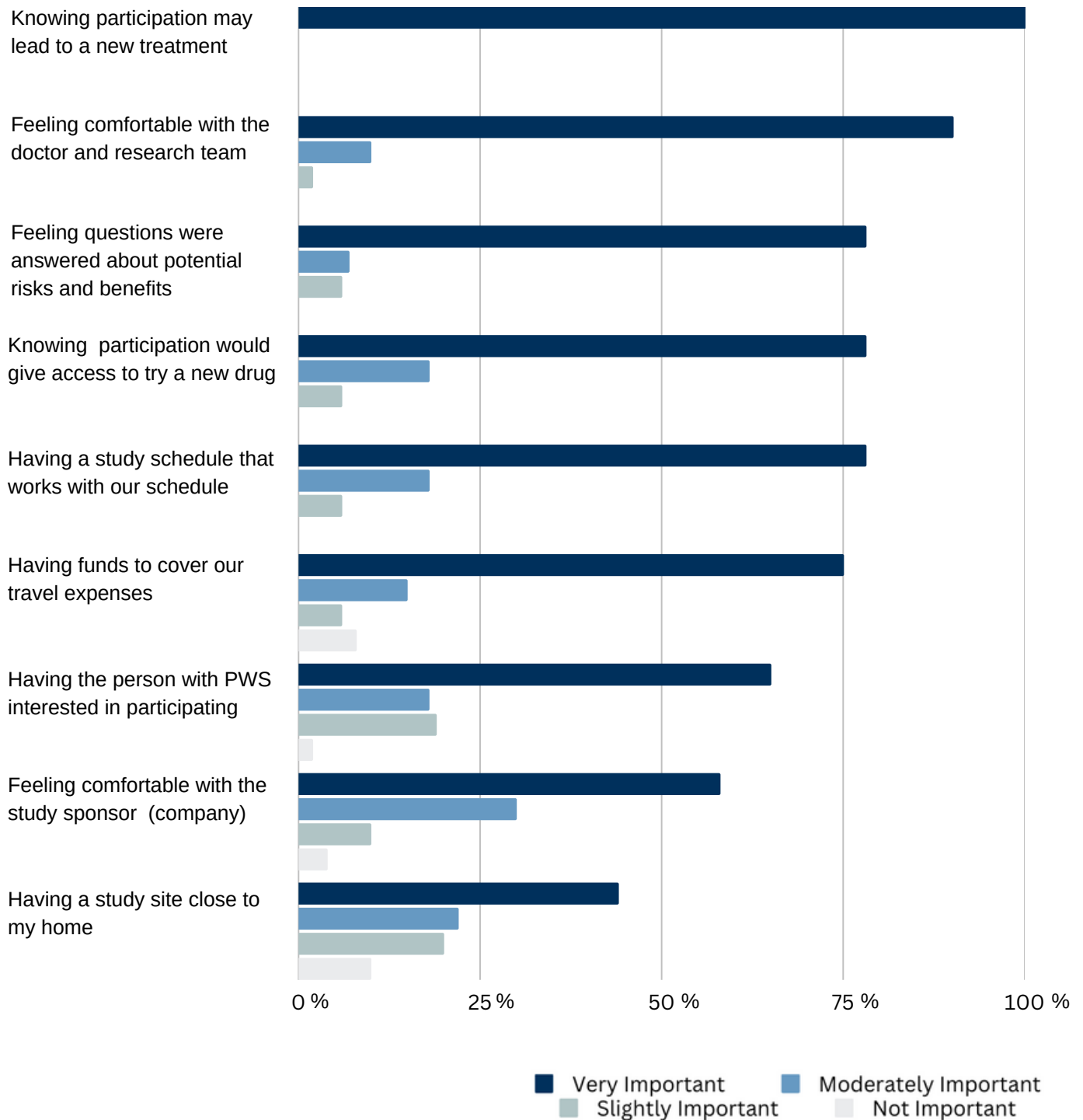
Who did you most rely on to give you information & make decisions about participating in a clinical trial?



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What were the most important factors that helped you decide to participate in a PWS clinical trial?

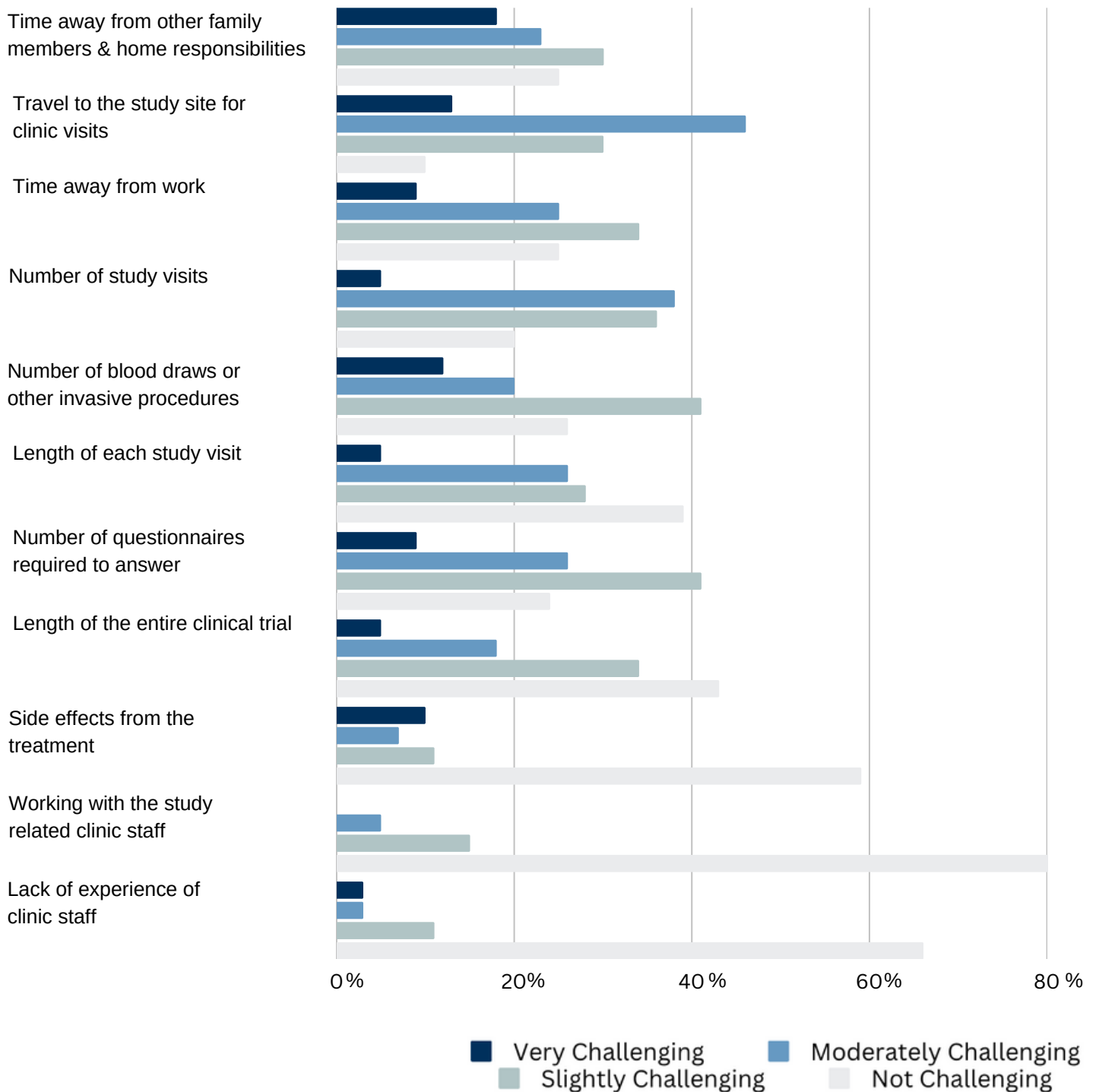


The possibility of trials leading to new treatments for PWS and feeling comfortable with the study team were most the important factors in deciding to participate.

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How challenging were the following aspects of the clinical trial for you and the person with PWS?

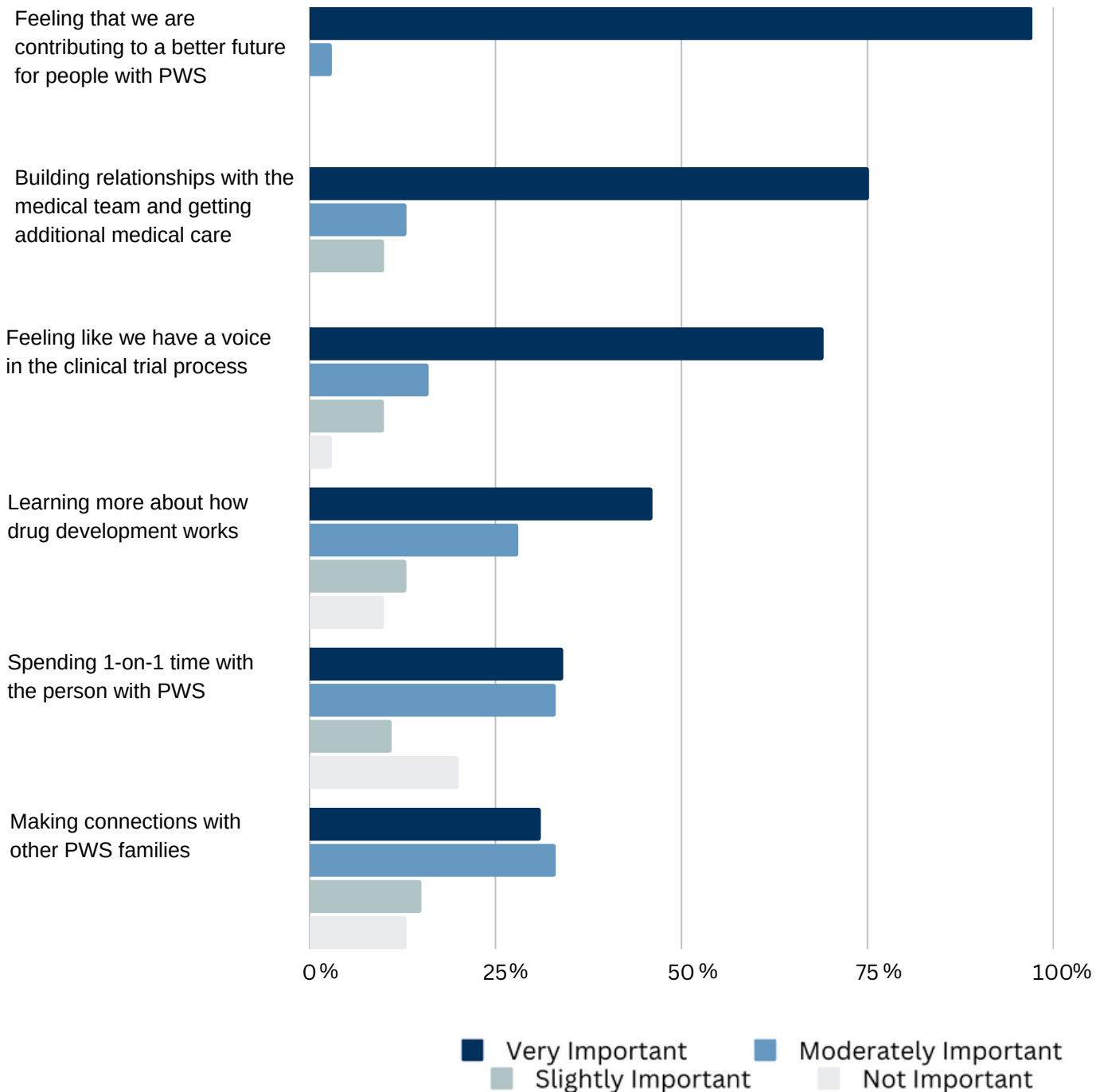


Travel to study visits, time away from other responsibilities and the number of study visits were among the most challenging aspects of participating in the trial

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Positive aspects of clinical trial participation:

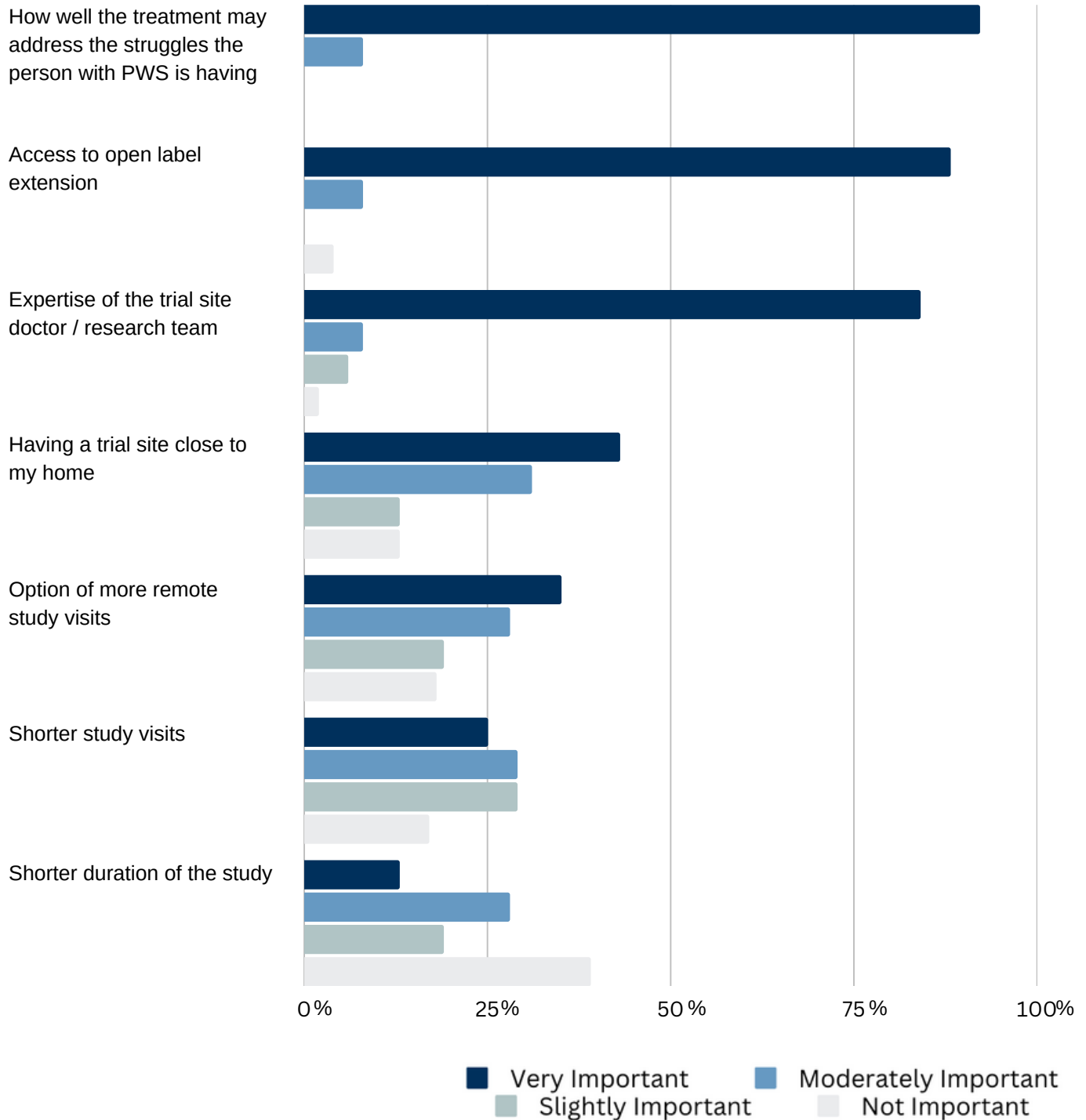


Caregivers reported that contributing to a better future for people with PWS; Building relationships with the clinical team and getting extra PWS care and having a voice in the clinical trial process as highly valuable and important aspects of participating in a trial.

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What factors would make you **more likely** to participate in future clinical trials?

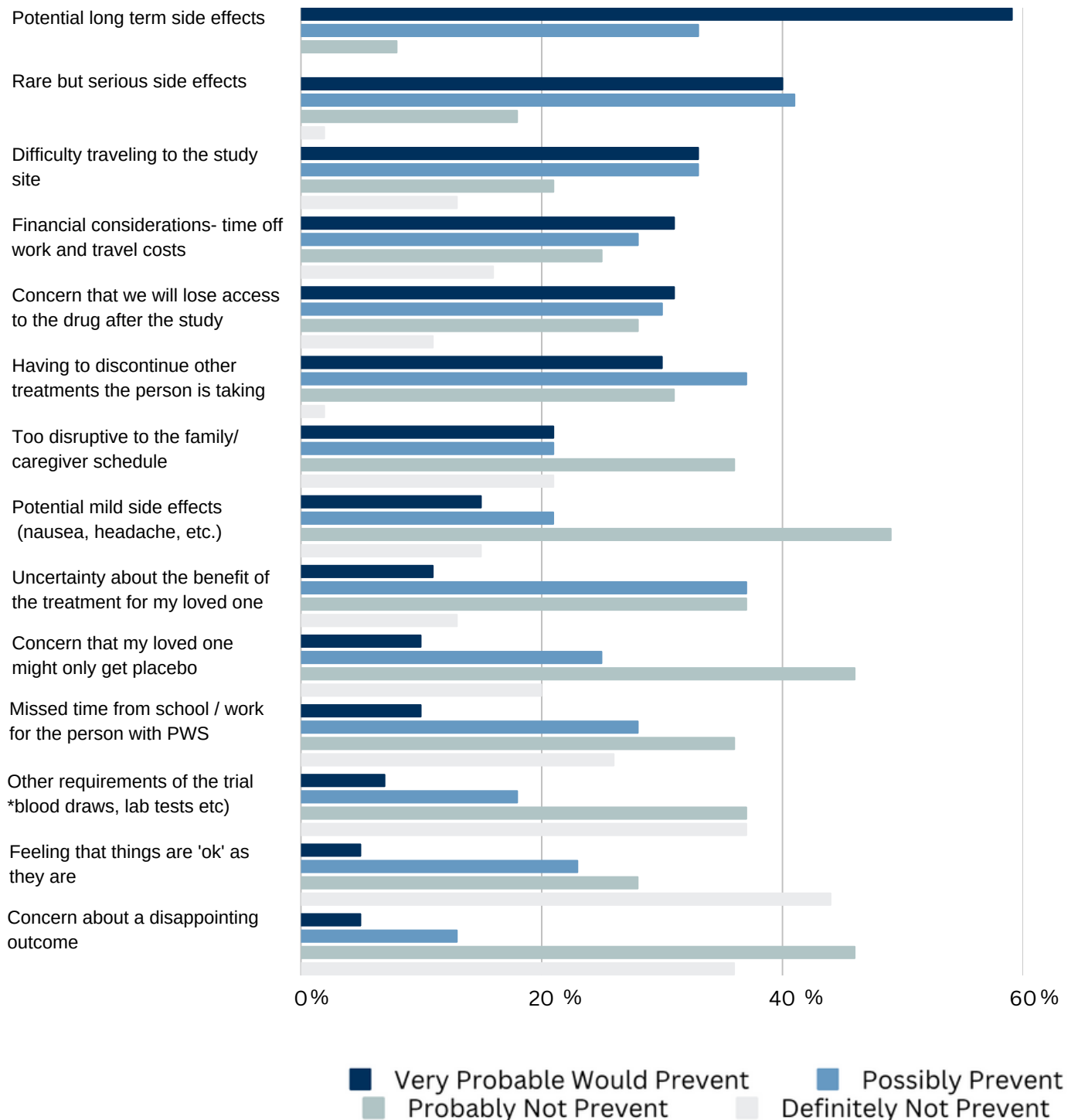


Caregivers noted that treatments that address their loved one's issues specifically, having access to open label extension and expertise of the study site team were all key factors that would lead them to sign up for another trial in the future.

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What factors would **prevent** you from participating in future PWS clinical trials?



Concerns about long-term and/or rare side effects and difficulty traveling and with financial costs associated with participating would limit their participation in future trials.

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Caregivers provided additional information about their PWS clinical trial experience and ways to improve trials for families. Key takeaways included:



Reduce the number of visits required:

"The less time required to travel and be on-site the better"

"Remote visits would be a huge plus, especially if we could miss less school"



Reduce the financial burden of participation:

"Provide full financial support for travel and ensure timely reimbursement"

"Have an easy process in place to access travel assistance"



Communicate test results whenever possible:

Clarify upfront what test results will be shared with the participant

Explain study results, when possible, to the caregiver



Communicate trial progress and changes:

"Be transparent with participants if and when a study might close early"

"It is really devastating when a trial closes early"



Always keep the patient first:

"It is a lot to ask PWS families to participate [in a trial]: we are already stressed out. We want to help and appreciate when the sites and the companies are supportive and aware of all the challenges of living with PWS."

Overall the survey showed we have an active, research-ready community that is eager to participate in trials that will help find new treatments to address the life-limiting challenges that their loved ones with PWS experience.