Health Equity in PWS Clinical Trials: PWS-CTC Spring 2025 Report

PWS Clinical Trial Consortium

A PUBLICATION BY THE PWS CLINICAL TRIALS CONSORTIUM

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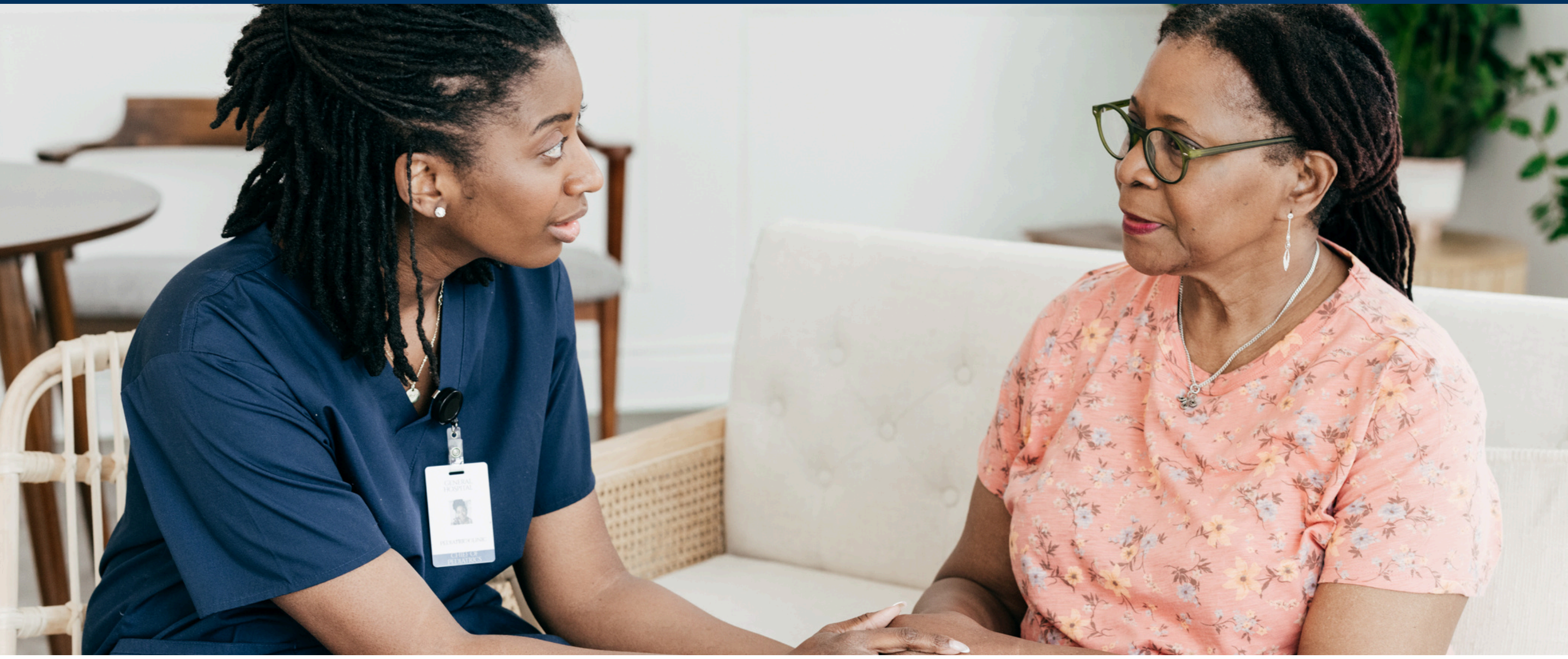

Recruiting a diverse and representative population for PWS clinical trials is essential to advancing health equity and ensuring that the safety and efficacy of new medical products is fully understood. The goal of the PWS-CTC Diversity and Inclusion project was to better understand efforts to recruit diverse participants to PWS clinical trials and identify opportunities to increase access for trial participation in the PWS space.

Project Activities

1. Trial Enrollment Comparisons

FPWR has gathered demographic data from completed PWS clinical trials. To better understand the current status of PWS clinical trial recruitment, this data is compared to Global PWS Registry demographics, US census data, and rare disease clinical trial enrollment data from the Rett and Fragile X communities.

2. Review of DEI Strategies and Best Practices

FPWR joined the Rare Disease Diversity Coalition (RDDC) in 2024 and has participated in RDDC working groups focused on strategies and best practices for increasing diversity in rare disease study populations. Please see page 4 for a summary of recommendations and strategies for recruitment of diverse populations for PWS clinical trials. These recommendations were gathered from multiple sources.

Race Data Comparisons

RACE	US CENSUS 2022	PWS CLINICAL TRIALS n=557	GLOBAL PWS REGISTRY n=1311	DAYBUE Rett Syndrome n=187	Trifinetide FragileX n=70
White	75.50%	77.20%	83.98%	92.00%	89.00%
Asian	6.3%	3.22%	4.5%	3.00%	1.00%
Black or African American	13.60%	3.78%	1.69%	1.00%	4.00%
Multi-Ethnic	3.00%	3.75%	7.63%	0	0
Native Hawaiian or Pacific Islander	0.30%	0.53%	0.23%	1.00%	0
Other	0	0.54%	1.37%	3.00%	6.00%
Not Reported	0	10.61%	0.31%	0	0

Ethnicity Data Comparisons

ETHNICY	US CENSUS 2022	PWS CLINICAL TRIALS n=557	GLOBAL PWS REGISTRY n=1311	DAYBUE Rett Syndrome n=187	Trifinetide FragileX n=70
Non-Hispanic Latino	81.00%	82.59%	82.38%	91.00%	91.43%
Hispanic Latino	19.00%	8.98%	9.52%	9.00%	7.14%
Other / Not Reported	0	8.44%	8.11%	0	1.43%

Summary: Recruitment of diverse samples for PWS clinical trials and other research studies is a priority for the field. PWS clinical trials and the Global PWS Registry appear to have somewhat low diversity compared to US census both in areas of race and ethnicity, especially in recruiting Black and Hispanic families for participation in clinical trials. However, PWS trials appear to have been more successful in recruiting diverse samples compared to some other rare disease trials. More focused efforts are needed towards recruiting families who are Hispanic, Black, and Asian as well as gathering more complete data as there is a significant amount of “unreported” race and ethnicity data in the PWS clinical trial study data. Additionally, it is not known whether Hispanic, Black, and Asian individuals with PWS are diagnosed at the same rate as White individuals. This likely has an impact on recruitment and could make it more challenging to recruit diverse samples for clinical trial studies. This issue should be addressed separately.

Improving Diversity & Access in PWS Clinical Trials
PWS Patient Advocacy Recommendations

- Address burden & barriers to access – reduce number of clinic visits, assist with transportation for all study activities, provide easy financial compensation to families for travel and missed work, add mobile & virtual options when appropriate, and offer families resources as needed.
- Develop trial sites in cities and areas with known greater diversity and where underrepresented communities live.
- Develop nontraditional trial sites – e.g., local pharmacies, urgent care clinics
- Recruit potential participants from outside large medical centers such as independent disease registries and smaller hospitals/community clinics
- Recruit study site clinicians who represent BIPOC and underrepresented communities
- Develop and support community clinical trial navigators who are part of diverse communities, work with patient advocacy organizations on initiatives to support individuals in these positions
- Develop trusted messenger programs to provide information and outreach to underrepresented and diverse communities – e.g., Yale Cultural Ambassador Program & Univ. of Maryland School of Pharmacy PATIENTS program.
- Leverage community voices such as faith-based leaders to share information about rare disease and clinical trial opportunities.
- Translate all materials into needed languages of the recruitment community and make language gender neutral.

Recommended Resources

- Rare Disease Diversity Coalition (RDDC) – Clinical Trials in Rare Disease working group
- Toward-a-National-Action-Plan-for-Achieving-Diversity-Clinical-Trials.Milken Institute, CTTI, MRCT Center of Brigham & Women’s Hospital
- FDA guidance document GFI.FDA DiversityActionPlan-6-24-24
- Guthy Jackson Charitable Foundation NMO Patient Day June 2024 – Fostering Diversity YouTube