Patient Perspective in Endometriosis and Clinical Trial Barriers

Emily Mulvihill[1]; Dawn Bates[1]; Rebecca Nash[1]; Sheila Drakeley[1]; Nikoletta Sternbach[1]

[1]Oracle Life Sciences, Austin, TX

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Introduction

Endometriosis is an under reported disease of unknown etiology. It is estimated to affect more than 11% of American women (6.5 million). Diagnosis can be difficult, with healthcare providers taking multiple approaches such as pelvic exam, image testing, and surgery[1]. Treatment for the pain of endometriosis include hormonal therapy (i.e. Oral contraceptives, Danazol), pain medicine or surgical treatments[2,3]. While endometriosis is actively being studied, barriers prevent participation in clinical trial research.

Our previous work[4] "Improving a Clinical Trial Design in Endometriosis Related Pain: Patient Engagement Research and Regulatory Impact" showed that endometriosis patients can provide meaningful and significant input on trial design.

Such input can lead to important and impactful adjustments, improvements to the study, and reduced participation barriers. In this study, patients voiced barriers to clinical trial participation and expressed concern over exclusion of patients with stable medical conditions other than ERP, such as depression. Based on this patient feedback, clinical considerations, and key safety imperatives, a major regulatory agency accepted that a range of requirements in the clinical trial protocol be adjusted in that study[4].

"The endometrial biopsy would probably change my willingness to participate, because I've heard from many women with endometriosis, and they had to go through that. I heard it's really painful."

- Patient, Clinical Trial Design Study

Objective

The present study characterized the US adult endometriosis population on disease burden and willingness to participate in clinical studies.

Methods

A cross-sectional analysis using data from the 2022 US National Health and Wellness Survey (NHWS), an online patient-reported outcomes survey administered to adults in United States (N= 75,098) that is age-sex-, race/ethnicity representative of the general population.

PROs[5,6,7,8]: GAD-7, PHQ-9, RAND-36, PAM

Self-reporting ever experiencing endometrial symptoms and diagnosed by a physician (EndoDx) or never experiencing endometriosis (EndoNever).

EndoDx were stratified as prescription medication users (UseRx) not taking a prescription medication (NoRx).

Statistical Analysis:

Unadjusted comparisons of patient demographic and patient reported outcomes between groups were conducted using chi-square tests and ANOVA tests for categorical and continuous variables, respectively. Two-sided p-values <.05 were considered statistically significant.

Results

Patient Demographics and Health Characteristics:

Our analytical sample consisted of 14,766 women (aged 18-50), of whom 13,693 never experienced endometriosis while 410 experienced symptoms and were diagnosed with endometriosis. Among women with endometriosis in the US, women were college educated (45.6%) and insured (88.8%); 41.0% saw a gynecologist in the past six months.

Compared to women who never experienced endometriosis, women diagnosed with endometriosis have higher rates of comorbidities (Figure 2) and lack reliable access transportation (Figure 3).

Diagnosed women were more likely to experience symptoms of anxiety (GAD-7) and depression (PHQ-9) compared to women never experiencing endometriosis. They also report an overall worse health-related QoL, scoring significantly lower (worse) for both the physical and mental health composite T score (means) for the RAND-36.

Figure 1 - Race & Ethnicity

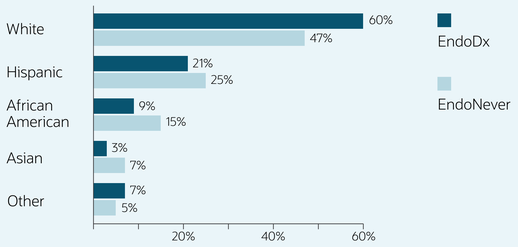


Figure 2 -Comorbidities

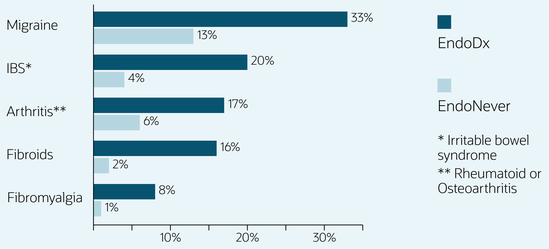
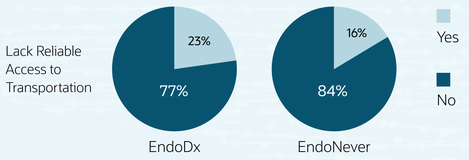


Figure 3 -Comorbidities Access to Transportation



For those diagnosed with endometriosis and experience symptoms, 139 use a prescription medication (UseRx) and 271 do not (NoRx).

Women taking an Rx reported greater severity of the disease compared NoRx (69.1% vs 24.4%).

Women taking an Rx indicated higher HCRU compared to NoRx (ER visits: 36.7% vs 26.9%; Hospitalized: 23.7% vs 14.4%).

The proportion of women with PAM level 4 (Maintaining behaviors and pushing further) is higher among those not taking an Rx compared to NoRx (19.7% vs 8.6%).

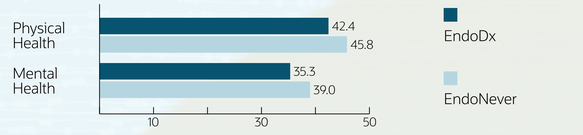
A greater proportion of women taking an Rx report they were willing to participate in a clinical trial compared to NoRx (51.1% vs. 47.6%).

Despite burden, almost **half (48.8%) of women** with endometriosis **were willing to participate in a clinical trial.**

"I would be signing up as soon as possible and just the encouragement would just be knowing that I'm helping possibly so many people get help. I wouldn't even necessarily be financially motivated. I would just love to know that thank God someone's listening out there and trying something."

- Patient, Clinical Trial Design Study

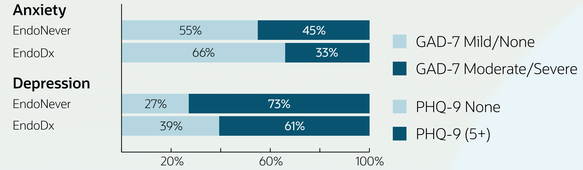
Figure 4 -Comorbidities RAND-36 Composite T Scores



"I just think that we spend so much physical and mental energy just trying to get someone to hear us, to acknowledge that it's not in our head, we're not hypochondriacs, we're not overreacting.

- Patient, Clinical Trial Design Study

Figure 5 -Comorbidities Mental Health

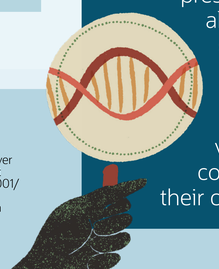


"I'm like, 'This is cool, but this isn't for me at all. I'm severely depressed. I have pretty severe anxiety. This PMDD [Premenstrual dysphoric disorder] diagnosis has turned my life into a tornado.' I don't know many people who just have endometriosis, but this is good for them."

- Patient, Clinical Trial Design Study

Conclusion

Women with high endometriosis disease burden are willing to participate in clinical trials. Clinical trial designers should consider the voiced needs of these patients, acknowledging their higher rates of comorbidities (i.e., depression and anxiety), lack of reliable transportation, lower activation in managing their health, and willingness to discontinue current prescription medication. This may involve allowing them to remain on medications or offering treatment alternatives for other comorbidities if their disease is stable; reimbursing patients for transportation or allowing them additional flexibility for visits (i.e., virtual, flexible window visits); and counseling about managing discontinuation of their current treatment regimen.



Limitations:

NHWS is patient-reported survey and responses are not verified by health care professionals, therefore there is potential recall bias and cannot be validated using claims or medical chart data. Due to NHWS being cross-sectional, associations can be measured, but causality cannot be assessed. Great care is taken to ensure NHWS is nationally representative, in terms of respondent age, gender, and race / ethnicity. However, this online endometriosis patient sample may not generalize to all endometriosis populations patients.

References:

1. Endometriosis | Office on Women's Health. (2021). https://www.womenshealth.gov/a-z-topics/endometriosis; 2. Buck, L.G.M., Hediger, M.L., Peterson, C.M., Croughan, M., Sundaram, R., Stanford, J., Chen, Z., et al. (2011). Incidence of endometriosis by study population and diagnostic method: the ENDO study. Fertility and Sterility; 96(2): 360-5; 3. U.S. Department of Health and Human Services. (2020.). What are the treatments for endometriosis?. Eunice Kennedy Shriver National Institute of Child Health and Human Development. https://www.nichd.nih.gov/health/topics/endometri/conditioninfo/treatment; 4. Nicklaus A. Improving a Clinical Trial Design in Endometriosis Related Pain: Patient Engagement Research and Regulatory Impact, World Endometriosis 2023; 5. GAD-7 Spitzer RL, Kroenke K, Williams JBW, Löwe B. A brief measure for assessing generalized anxiety disorder—The GAD-7. Arch Intern Med. 2006;166(10):1092–7. doi: 10.1001/archinte.166.10.1092; 6. PHQ-9 Kroenke, K., Spitzer, R.L., & Williams, J.B. (2001). The PHQ-9: validity of a brief depression severity measure. Journal of General Internal Medicine, 16606-613.; 7. RAND-36 Hays RD, Prince-Embury S, & Chen H. RAND-36 Health Status Inventory. San Antonio, TX: The Psychological Corporation; 1998; 8. PAM Hibbard JH, Mahoney ER, Stockard J, and Tusler M. Development and Testing of a Short Form of the Patient Activation Measure. Health Services Research. 2005;40:1918-1930. https://doi.org/10.1111/j.1475-6773.2005.00438