Society for Women's Health Research

February 16, 2018

Submitted electronically to: *https://www.regulations.gov*

Theresa M. Mullin, PhD
Director, Office of Strategic Programs
Center for Drug Evaluation and Research
Food and Drug Administration

Re: Docket No. FDA-2017-N-5896 for "Patient-Focused Drug Development: Guidance 1 – Collecting Comprehensive and Representative Input; Public Workshop; Request for Comments"

Dear Dr. Mullin:

The Society for Women's Health Research (SWHR®) commends the Food and Drug Administration (FDA) for conducting the December 18, 2017, Patient-Focused Drug Development (PFDD) public workshop on methodological approaches for collecting comprehensive and representative input from patients and caregivers. The agency has led significant, collaborative work to date on PFDD, and SWHR is pleased to provide these comments.

SWHR, a nonprofit organization based in Washington, DC, is widely recognized as a national thought leader in promoting research on biological differences in disease and eliminating imbalances in care for women through science, advocacy, and education. SWHR strongly supports the implementation of the Prescription Drug User Fee Act (PDUFA) VI and provisions of the 21st Century Cures Act that integrate the patient voice in drug development and regulatory decision-making.

Patient-focused drug development is both promising and exciting because it is a recognition of the inherent value of engaging patients in a process to identify outcomes that matter to them—such as quality of life, productivity, risk of disability—as well as the need to measure the impact of new treatments on those outcomes. The patient-centered care model appropriately recognizes patients as individuals with different characteristics, needs, and preferred outcomes.

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The unique considerations of women as patients, caregivers, and family decision-makers across the lifespan should be examined and addressed in the PFDD construct.

Women comprise more than half (51%) of the U.S. population[1] and provide the majority of caregiving. An estimated 66% of caregivers are female.[2] Women play many roles while caregiving: hands-on health provider, case manager, friend, companion, surrogate decision-maker and advocate.[3] Furthermore, women make more than 70% of health care spending decisions.

Both sex and gender are important factors to consider to achieve sufficient representation of the target population.

A number of common conditions, disorders, and diseases are unique to women or occur disproportionately in women. Certain conditions and diseases are associated with different risk factors in women than in men. Based on a large and growing evidence base, it is now commonly accepted that there is a biological basis for sex differences in health and that cellular biology is sex-specific. In fact, scientists are required to consider biological sex and gender as variables in federally funded research.

- *Biological sex* is defined as the classification of living things as female or male based on the complement of sex chromosomes and the presence of reproductive organs.[4]
- *Gender* refers to complex psychosocial construct that takes into account biology but also the influences of society and environment.[5]

The study of sex and gender differences is leading to important discoveries of how women and men differ in fundamental ways and how these differences affect disease risk, symptoms, diagnostic sensitivity and specificity, and response to therapy.

SWHR is pleased that Figure 7 (Factors to Consider to Achieve Sufficient Representation) on page 23 of the discussion document includes "sex" in the description of socioeconomic and demographic background. However, "gender"—a type of patient experience data—is missing and should also be included.

"Biological sex" and "gender" should be added to the PFDD draft glossary.

Establishing standardized nomenclature and terminologies is paramount to having shared understanding of principles and meaningful dialogue pertaining to PFDD. We applaud FDA for developing a robust draft glossary of terms as an attachment to the discussion document.

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[1] Kaiser Family Foundation State Health Facts. Population Distribution by Gender. 2016 Timeframe. https://www.kff.org/other/state-indicator/distribution-by-gender/?currentTimeframe=0&sortModel=%7B%22colId%22:%22Location%22,%22sort%22:%22asc%22%7D. Accessed February 2018.
[2] Family Caregiver Alliance. Women and Caregiving: Facts and Figures. https://www.caregiver.org/women-and-caregiving-facts-and-figures. Accessed February 2018.
[3] Ibid.
[4] Exploring the Biological Considerations to Human Health: Does Sex Matter? In: Wizemann TM, editor; Pardue ML, editor, Washington, DC: The National Academies Press, 2001.
[5] Ibid.

SWHR recommends that "biological sex" and "gender" be added to the PFDD glossary using the cited definitions provided above.

Further, while we understand that the terms in the draft glossary "have been defined specifically for the context of medical product development and regulatory decision-making,"[6] we expect a wide variety of stakeholders to use this glossary. As such, we encourage FDA to make the glossary widely accessible and identify opportunities to raise the awareness about its purpose and utility in PFDD.

Standards for patient data collection and management are vital and should be developed in a collaborative manner.

SWHR strongly supports the development of consensus-driven standards for patient data collection, submission, and management. To be reliable and effective, such standards must be based on methodologically sound approaches that accommodate the distinct and varying perspectives of patients on the value of interventions, while simultaneously collecting patient experience data that is relevant, objective, accurate, and representative of the target population. Standards should be flexible with the capacity to evolve over time. As stakeholders gain more experience with data collection, submission, and management, standards and processes may need to be revisited and revised.

Given the many factors that must be considered in standardizing data collection activities, we agree with the conclusions statement of the discussion document (Section 5) that the "proposed methods ... serve only as a basis for dialogue in the evolving and growing area of the science of patient input."[7] Along those lines, we encourage FDA to continue to facilitate ongoing, constructive dialogue with both industry and patient advocacy organizations that allows for a transparent and organized process for developing standards for the collection, submission, and management of patient experience data.

Harmonization across the series of guidance documents is vital to PFDD success.

SWHR supports the stepwise manner FDA is taking to develop the series of four PFDD guidance documents. Because the topics and questions to be addressed in each document are closely interrelated, "harmonization" across the guidance series should be an overarching consideration and guiding principle. As FDA moves forward with finalizing this guidance while simultaneously initiating development of the other three guidance documents, we encourage the agency to evaluate existing resources, methods, and tools that can be used to evolve the science of patient input. Researchers and the pharmaceutical industry have deep experience and expertise using patient-reported outcomes (PROs) and PRO measures (PROMs) as a means to ensure patient perspectives are directly captured as part of assessing treatment efficacy and outcomes of interest to patients. FDA should assess opportunities for using

[6] FDA, Patient-Focused Drug Development Public Workshop on Guidance 1, Attachment to Discussion Document, Draft Standardized Nomenclature and Terminologies for the Series of FDA PFDD Guidances (Glossary), Workshop Date December 18, 2017, Page 2.
[7] FDA, Patient-Focused Drug Development Public Workshop on Guidance 1 – Collecting Comprehensive and Representative Input: Discussion Document, Workshop Date December 18, 2017, Page 34.

reliable and validated PROs/PROMs as a source of representative input, such as the Menopause-Specific Quality of Life (MENQOL) Questionnaire for assessing overall menopause quality of life.[8] In a study that examined the psychometric properties of the MENQOL in a population of breast cancer survivors, the instrument performed nearly as well in this subgroup as the target population of women experiencing natural menopause.[9]

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SWHR commends FDA for its important work on PFDD to date, and we appreciate the opportunity to comment on Docket No. FDA-2017-N-5896 for "Patient-Focused Drug Development: Guidance 1 – Collecting Comprehensive and Representative Input; Public Workshop; Request for Comments." We hope you will take our input on this important topic into consideration.

If you have questions, please contact Sarah Wells Kocsis, Vice President of Public Policy, at 202.496.5003 or swellskocsis@swhr.org.

Sincerely,

Amy M. Miller, PhD
President and Chief Executive Officer
Society for Women's Health Research

[8] Hilditch, John, et al. The Menopause-Specific Quality of Life Questionnaire. Maturitas. 1996; 24: 161-175.
[9] Radtke Jill, et al. The Menopause-Specific Quality of Life (MENQOL) Questionnaire: Psychometric Evaluation among Breast Cancer Survivors. Menopause. 2011 Mar; 18(3): 289-295.