Society for Women's Health Research

December 14, 2018

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

The Honorable Scott Gottlieb, MD
Commissioner
Food and Drug Administration

Re: Docket No. FDA-2018-N-2455-0001 for Patient-Focused Drug Development Guidance

Dear Dr. Gottlieb:

The Society for Women's Health Research (SWHR) commends the Food and Drug Administration (FDA) for conducting the October 15 and 16, 2018, Patient-Focused Drug Development (PFDD) public workshop on methods to identify what is important to patients and select, develop, or modify-fit-for-purpose clinical outcome assessments.

The agency continues to lead significant, collaborative work on PFDD, and SWHR is pleased to provide these comments.

SWHR is an education and advocacy thought leader dedicated to promoting research on biological differences in disease and improving women's health through science, policy, 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 into drug development and regulatory decision-making.

Patient-focused drug development is promising and exciting because it recognizes 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.

FDA's PFDD Program should address the unique considerations of women as patients, caregivers, and family decision-makers across the lifespan.

Women comprise more than half (51%) of the U.S. population[1] and provide the majority of caregiving. An estimated 66% of

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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.

FDA's PFDD Program should address patient population diversity (including sex and gender) to the extent possible.

A number of common conditions, disorders, and diseases are unique to women or occur disproportionately in women. Further, many conditions and diseases affect women and men differently. There is a biological basis for sex differences in health that begins in early development and can be found even at the cellular level. In fact, scientists are required to consider sex and gender as variables in federally funded research.

Sex and gender play critical roles in the risk, pathophysiology, presentation, diagnosis, treatment, and management of disease. As defined by the Institute of Medicine:

- *Sex* refers to the classification of living things according to reproductive organs and functions assigned by chromosomal complement.[4]
- *Gender* refers to the social, cultural, and environmental influences on the biological factors of women or men. Gender is rooted in biology and shaped by environment and experience.[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.

FDA's PFDD glossary should add the terms "sex" and "gender" as defined by the Institute of Medicine.

Establishing standardized terminology is paramount to achieving shared understanding of principles and meaningful dialogue pertaining to PFDD. We applaud FDA for developing a robust PFDD glossary of terms[6] and evolving it as needed.

SWHR requests that "sex" and "gender" be added to the PFDD glossary using the cited definitions published by the Institute of Medicine.

Further, while we understand that the terms in the glossary "have been defined specifically for the context of medical product development and regulatory decision-making,"[7] we expect a wide variety of stakeholders to use the FDA PFDD 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.

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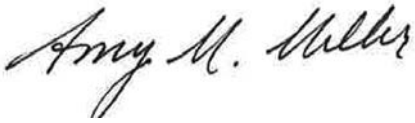
SWHR commends FDA for its important work on PFDD to date, and we appreciate the opportunity to comment on Docket No. FDA-2018-N-2455-0001 for "Patient-Focused Drug Development: Methods to Identify What is Important to Patients and Select, Develop, or Modify Fit-for-Purpose Clinical Outcome Assessments." We hope you will take our input and requests 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
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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] Institute of Medicine. Exploring the Biological Considerations to Human Health: Does Sex Matter? Washington, DC: The National Academies Press, 2001.
[5] Ibid.
[6] US Food and Drug Administration. Patient-Focused Drug Development: Collecting Comprehensive and Representative Input. Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders (Draft Guidance 1). June 2018.
[7] 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.