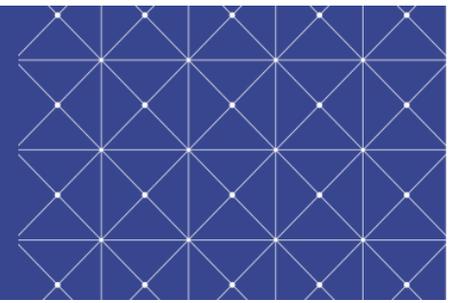


POLICY PRINCIPLES: HEALTH CARE VALUE ASSESSMENT



Background

The U.S. health care system is undergoing transformative changes in the way that it delivers and pays for care. The value of health care interventions and treatments cannot be determined without proper assessment and measurement.

Value frameworks assess evidence from clinical and economic data to inform decision-making about health care interventions for a range of audiences including patients, health care providers, and entities that pay for health care, such as insurance companies and federal agencies.¹

SWHR is committed to ensuring value frameworks are appropriately designed and used to provide appropriate access to innovative new therapies and interventions for women and to achieve optimal health outcomes for women as patients, caregivers, and health care decision-makers for themselves and their families.

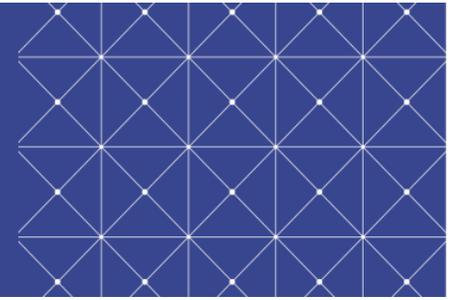
- Women comprise more than half (51%) of the U.S. population.²
- Women provide the majority of caregiving.
 - Nearly 70% of caregivers are female.³
 - Women assume multiple roles while caregiving: hands-on caregiver, case manager, companion, decision-maker, and advocate.
- Women make more than 80% of health care spending decisions.⁴

SWHR has conceived a set of principles to help ensure value frameworks and assessments reflect factors relevant to women and the ongoing improvement of their health. This includes making sure that value frameworks account for both patient population diversity (including sex and gender) and have the infrastructure and analytic capability to evaluate data that matters to women.

About SWHR

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. Founded in 1990 by a group of physicians, medical researchers, and health advocates, SWHR championed the framework for the scientific discipline of sex-based biology, which advocates for the inclusion of female subjects in clinical trials and analyzes the differences between women and men in relation to disease. Today, SWHR continues to lead the way in correcting imbalances in health care for women by identifying research gaps in women's health and bringing attention to a variety of diseases and conditions that disproportionately or differently affect women.

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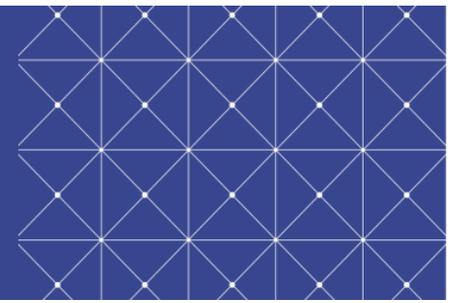
SWHR Value Assessment Principles

1. Value assessments should account for *diversity in patients (including sex and gender)* for a given disease state by analyzing data that represents relevant patient populations and subgroups.
 - *Sex and gender* play critical roles in the risk, pathophysiology, presentation, diagnosis, treatment, and management of disease.
 - *Sex* refers to the classification of living things according to reproductive organs and functions assigned by chromosomal complement.⁵
 - *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.⁶
 - When women are underrepresented in clinical trials, outcomes from predominantly male cohorts drive clinical guidelines that are not sex-specific.⁷
 - Increased 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.
 - Biological and physiological differences and hormonal fluctuations have been shown to play a role in the rate of drug absorption, distribution, metabolism, and elimination, resulting in different drug responses in women and men.⁸
2. Value assessments should acknowledge the *full spectrum of treatment options* for a given medical condition.
 - Patient subpopulations can differ in their response to therapy (i.e., heterogeneity of treatment effect). Therefore, value assessments for new therapies should take into consideration patients who, for example, cannot tolerate currently available therapies, are contraindicated for these therapies, have heterogeneous responses to these therapies, or for whom these therapies are ineffective.
 - Value assessments should not focus exclusively on medications or one type of medical intervention but should instead consider all available evidence-based options within the health care system.
 - Some options may include interventions not regulated by the U.S. Food and Drug Administration (FDA), such as evidenced-based behavioral therapies and lifestyle interventions.

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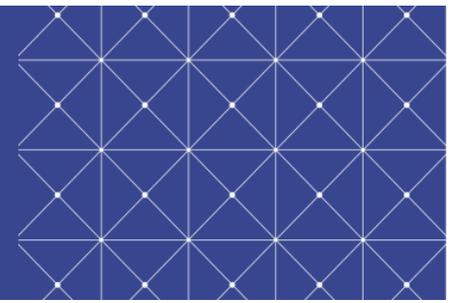
3. In addition to measuring clinical outcomes, value assessment frameworks should account for *what matters most to patients, caregivers, and society*.
- Value measurement should include a broad array of factors to provide a comprehensive snapshot of a treatment's value. These factors should be quantitatively accounted for in value assessment cost-effectiveness methodologies.
 - Patient values vary and change across patient populations.
 - At a population level, patient values are shaped by social, religious, and cultural factors and the health care environment in which patients live.
 - At an individual level, treatment-based values are influenced by age, sex and gender, education, family and friends, attitude to work and career, and personal finances, among other factors.⁹
 - Burden of illness factors that are important to women include (but are not limited to):
 - Survival
 - Ability to function/work
 - Presenteeism
 - Absenteeism
 - Employment disability
 - Quality of life
 - Physical and social well-being
 - Pain or discomfort
 - Levels of disease burden and progression
 - Comorbid conditions or concomitant medications
 - Caregiver burden¹⁰
 - Permanent difficulty, stress, or negative experiences resulting from providing care¹¹
 - Physical, emotional, and financial cost of the caregiving
 - Limitations in treatment
 - None (i.e., a treatment does not exist for a particular condition or disease)
 - Limited options (i.e., there have been few innovations in the disease state, the products on the market are contraindicated for a subset or subsets of patients, or available therapy does not meet the patient's preference)
 - Values are dynamic and change over time as patients' individual circumstances and experience of illness and treatment evolve throughout the course of disease:
 - Shifts in prognosis

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- Severity of illness
 - Comorbidities (e.g., obesity)
 - Available treatment/Palliative options
 - Life events¹² (e.g., pregnancy, menopause)
- Value assessments should evaluate the impact that utilization management (UM) procedures such as step therapy and fail first have on patients' health outcomes and system costs compared to open access.
4. Value assessments should take into consideration the long-term benefits of a therapy in addition to short-term benefits. Focusing only on short-term outcomes may overlook important clinical benefits that can only be measured over a significant time horizon. Additionally, incorporating long-term outcomes will enable value assessments to account for the full value of a therapy or intervention, particularly as additional evidence continues to emerge post-approval.
 5. Value assessments should use a range of high-quality evidence to demonstrate an improvement in outcomes.
 - Real-world evidence (RWE), not just data collected from controlled clinical trials, should be considered.
 - [RWE](#) is derived from data collected during routine health care practice (such as electronic health records, claims and billing activities, or product and disease registries).
 - RWE is often collected *after* a new therapy is already on the market and being used by patients.
 - Data on caregivers — the majority of whom are women — should be considered, even though this information is not typically collected in clinical trials.
 - Pertinent evidence from stakeholders that may not be available in published literature should be considered.
 6. Value assessment organizations should provide ample opportunities for *stakeholder engagement* to ensure their input is both acknowledged and meaningfully incorporated into assessments.
 - Proposed assessment topics, processes, and timelines should be announced in advance to allow for participation by stakeholders, whose resources may be limited.

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- Sufficient time should be allocated for stakeholders to review materials and submit comments in various stages throughout the assessment process.
 - Timeframes for stakeholder input on value assessment reviews should be commensurate with established and customary timeframes for other stakeholder review timeframes (i.e., federal government public comment periods typically are not less than 30 days and frequently a minimum of 60 days).
 - Stakeholders who have direct experience and expertise with a particular illness and its burden should be appropriately represented on value assessment panels and committees tasked with making determinations about a treatment's value. These stakeholders include:
 - Patients who are diagnosed with the disease/condition under review
 - Health care professionals who actively treat patients with the disease/condition under review
 - Caregivers who assist patients with care needs for the disease/condition under review
 - Assessments should be regularly updated to account for new innovation and other changes in the evidence base.
7. Value assessment processes, methodologies, and results should be *transparent* to all stakeholders.
- Explanation of assessment criteria, methodologies, and assumptions should be understandable to patients and other stakeholders.
 - Detailed information about how stakeholder input was considered, addressed, or incorporated into the assessment should be clearly communicated.
 - Models and data used should be made publicly available to allow for research to be analyzed and results replicated by others.
8. The *intended use* of value assessment frameworks, and by whom, should be clearly articulated.
- To avoid misuse, intended audiences and the decision-making on health care that value assessments aim to support should be clearly stated.
 - For example, a value assessment designed for shared decision-making between patients and their health care providers may not be designed for use by payers.¹³
 - Value assessments should not be used to prevent patients and their physicians from making evidence-based decisions that are tailored to the specific needs of individual patients.

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- ⁸ US Food and Drug Administration Drug Trial Snapshots. www.fda.gov/Drugs/InformationOnDrugs/ucm412998.htm
- ⁹ Addario et al. Patient value: Perspectives from the advocacy community. Health Expectations. 2017:1-7. <https://onlinelibrary.wiley.com/doi/full/10.1111/hex.12628>.
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- ¹¹ Simon BS, Budó MDL, Garcia RP, et al. Social support network to the caregiving family of an individual with a chronic disease: integrative review. Journal of Nursing UFPE on line [JNUOL/DOI: 10.5205/01012007] 2013;7:4243–4250. [Google Scholar]
- ¹² Addario et al. Patient value: Perspectives from the advocacy community. Health Expectations. 2017:1-7. <https://onlinelibrary.wiley.com/doi/full/10.1111/hex.12628>.
- ¹³ National Pharmaceutical Council. Guiding Practices for Patient-Centered Value Assessment.