

Clinical Decision-making in the Face of Scientific Uncertainty: Hormone Replacement Therapy as an Example

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There is widespread variation in the prescribing patterns of postmenopausal hormone replacement therapy. While some degree of variation is expected, the systematic variation according to geographic region, physician gender, and medical specialty raises questions about how clinical decisions are made. This paper explores the determinants of these practice patterns, specifically the contribution of patients' preferences, scientific uncertainty, and physicians' recommendations. A role for collaborative decision-making is described and the use of decision-support tools is discussed. The primary care

setting is proposed as the ideal context in which to study collaborative decision-making. Additional research is needed to more fully elucidate the value of collaborative decision-making with respect to clinical and quality-of-life outcomes, patient satisfaction with decision-making, and costs.

Key words. Clinical decision-making; primary health care; hormone replacement therapy; patient satisfaction; physicians' practice patterns.

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Medical practice variation has been demonstrated for many medical and surgical therapies.¹ For example, rates of estrogen prescriptions vary threefold across regions of the United States,² and vary significantly with the specialty and gender of the prescribing physician.³ Some variation in the use of hormone replacement therapy (HRT) is expected, as the decision involves more than one treatment option and varying patient preferences for available options.⁴ What is unexpected is the systematic variation in prescribing patterns according to geographic region, physician gender, and other nonclinical factors.^{2,3}

This paper examines the role of patients' preferences in determining the appropriateness of treatment using the example of HRT for disease prevention. It also explores

the role of scientific uncertainty and physicians' recommendations and discusses the ways in which the context of primary care can facilitate the incorporation of patients' preferences into clinical decision-making. The challenges of collaborative decision-making are discussed, and the tools for assisting providers and patients in making collaborative decisions are described.

The Role of Patient Preferences in Postmenopausal Hormone Replacement Therapy Decision-making

As women grow older, they face increasing risks of two important diseases: coronary artery disease and osteoporosis.⁵ Long-term estrogen replacement therapy has been shown to reduce the risk of heart disease by about 50% (M. J. Stampfer, personal communication, 1995) and reduce the risk of hip fractures by about 25%.⁵ Progestins are routinely prescribed with estrogens for women who still have a uterus to offset the risk of endometrial cancer that is associated with unopposed estrogen.⁵ Progestins

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probably somewhat attenuate the benefit of estrogen in preventing heart disease but do not appear to significantly reduce the benefit estrogen affords in preventing osteoporosis.⁵ That is the good news.

The bad news, or at least the news that may make the decision difficult, is that long-term estrogen therapy may increase the risk of breast cancer by between 25%⁵ and 45%.⁶ Moreover, there is some uncertainty about the effects of estrogen on the heart because most studies looking at the efficacy of hormones were not randomized.⁵ There is even greater uncertainty about the effects of estrogen on the breast. In general, studies of the relationship between postmenopausal hormone use and breast cancer have also been observational and have had inconsistent results.^{5,6,7}

There are nonhormonal alternatives to reducing the risk of osteoporosis^{8,9} and heart disease.¹⁰ Unfortunately, scientific knowledge about the comparative efficacy of estrogen and nonhormonal options (or combinations of options) is incomplete. The challenge for the patient is to weigh the potential harms against the possible benefits of hormone replacement therapy in the context of considerable scientific uncertainty.

There are other concerns as well. Patients vary greatly in regard to baseline risks for the diseases in question as well as in their views about the relative importance of preventing osteoporosis or heart disease as opposed to avoiding cancer.¹¹ Patients vary even in their willingness to take pills,¹² with many preferring non-pharmacological approaches.

Most physicians would agree that the decision whether to take hormones for prevention should be individualized. It would follow that patterns of hormone use would vary, yet the health services literature shows surprising practice patterns. Women on the West Coast have been shown to be three times as likely as women on the East Coast to use estrogen.² In Boston, women who have female internists were 11 times more likely to use HRT than were women who have male internists.³ Do women's views systematically differ according to where they live or by the gender of their physician? Perhaps, but probably not as much as the practice variation might suggest.

The Role of Physicians' Recommendations in Clinical Decisions

Historically, physicians' recommendations have been important determinants of treatment decisions.¹³ Obviously, physicians' recommendations should be based, to the extent possible, on scientific evidence. It is believed that physicians' recommendations are sensitive to the social and economic environment.¹⁴ It is also believed that

physicians' recommendations are sensitive to the level of certainty about the best course of action for a particular clinical situation. When the data are inconsistent and controversial, physicians' thresholds to recommend therapy vary.¹⁵

Consider the quality of data on the efficacy of long-term HRT on reducing the risk of heart disease. Although the observational data point toward a benefit in reducing the risk of heart disease, professionals have mixed reactions.¹⁶ The data about the relationship between long-term estrogen and breast cancer are even more controversial.⁵ In the setting of scientific uncertainty about the risks and benefits of HRT, physicians' recommendations are likely to vary.¹⁵

Physician uncertainty reaches beyond interpreting the data on the risks and benefits of therapy. Providers also express uncertainty about how to screen candidates for therapy and how to monitor patients once hormones have been prescribed.¹⁷ Physicians also have different attitudes about prescribing a potentially harmful medication to healthy women for prevention of future disease. When asked, "Do you consider that even a small increase in the risk of cancer, either of the breast or uterus, would preclude the use of unopposed oestrogens, regardless of any benefit to cardiovascular disease?" British physicians were divided.¹⁸ Uncertainty about whether the risks outweigh the benefits, from the physicians' perspective, also undoubtedly influences physicians' recommendations.

Unless they inquire directly, physicians may also be uncertain about what matters to their patients. The attitudes and preferences of physicians and perimenopausal women regarding health outcomes associated with estrogen replacement are known to differ.¹¹ This certainly underscores the need to develop practical methods to help elicit patients' attitudes and preferences about their different health states.¹⁹ The literature increasingly suggests that patients are better suited than physicians to judge the value of health states, particularly when quality-of-life issues are concerned.²⁰ Moreover, it has been suggested that outcomes would be improved if treatment decisions matched patients' values.²¹

The Value of Collaborative Decision-making

More direct evidence suggests that having a choice may lead to improved outcomes. For example, studies have suggested that having a choice between surgical alternatives for early-stage breast cancer may be psychologically beneficial to patients.^{22,23} Involving patients directly in their care has also resulted in better outcomes for patients with peptic ulcer disease²⁴ and diabetes.²⁵ While prelimi-

nary results suggest that promoting collaborative decision-making and encouraging patients to be more active participants in their care may lead to superior outcomes, there are still many unanswered questions about these activities.

Research suggests that patients' desire for information and involvement in decision-making is not universal, nor are the two characteristics necessarily correlated. For example, there are patients who want detailed information about their treatment who do not necessarily desire to actively participate in the decision-making process.^{26,27} Further study is also needed to determine whether patients' decision-making styles are durable and their preferences consistent across clinical scenarios.

There is also much to be learned about decision-making styles of physicians and about how physicians and patients should be paired for the best results. From the physicians' perspective, there is also much to be learned about *how* to collaborate. Merely giving information may not be enough. It may be inappropriate to put the decision entirely in the patient's hands without guidance. Interpreting information, supporting the patient, and even making the final decision when asked to do so are all consistent with the idea of collaborative decision-making. The physician-patient dialogue can be complex and variable.²⁸

There is also much to be learned about the predictive value of patient preferences. When examined at 3 years after their surgery, breast cancer patients who were treated by surgeons who offered a choice between breast-conserving surgery and mastectomy showed less psychiatric morbidity than women whose surgeons favored mastectomy.²⁹ When asked to reflect on the process of having been given a choice, about one half of the patients had positive reactions, some were uncertain, and only about one in five had reservations about the process.²⁹ Key questions raised by the study include: (1) how was the choice presented? (2) how was the decision actually made? and (3) what were the retrospective reactions of the women who had *not* been given a choice? Eighteen states have enacted legislation that promote disclosure of information about treatment options for breast cancer, an intervention that has high face validity but has not been well tested.³⁰ Such legislative initiatives make the need to study these issues even more pressing.

Facilitating the Practice and Study of Collaborative Decision-making in Primary Care

Although the principles of collaborative decision-making can be adopted by any clinical setting, the process of

collaborative decision-making can probably be better introduced, disseminated, and studied in the primary care setting where, ideally, care is first contact, longitudinal, comprehensive, and coordinated.³¹

It takes time for patients and physicians to learn how to collaborate, and presumably, in well-coordinated health care systems, patients will have first and more frequent contact with their primary care provider than with other types of providers. The primary care setting would be the ideal training ground for the collaborative decision-making process. The longitudinal nature of the primary care patient-physician relationship should foster this learning experience and provide a context for studying the process over time. Another defining characteristic of primary care is that it is coordinated. Better integration of the primary care provider's input into subspecialty care decisions might be beneficial, although further study is necessary.

The primary care setting is well suited for studying other issues surrounding collaborative decision-making. One of the key questions is which decisions result in superior outcomes: those made jointly by the patient and physician, those made more by the patient than the provider, or those made primarily by the physician. While preliminary research looks promising and the face validity of patient empowerment and collaborative decision-making is high, there are potential risks to the patient, such as anxiety during the decision-making process or regret when an adverse outcome follows a decision that the patient heavily influenced.¹⁹

It is possible that collaborative decision-making will require more time for discussion of pros and cons than is allocated in current practice. Lack of time has been cited as a barrier to discussing HRT.¹⁷ High-quality collaborative decision-making may also require that both physicians and patients have convenient access to current information about the risks and benefits of therapy.

There is a wide range of possible benefits associated with collaborative decision-making. These include a higher likelihood of receiving a therapy that is concordant with the values and preferences of the individual patient.²¹ In addition to experiencing improved clinical and quality-of-life outcomes, being informed about the available options and involved in the decision may result in higher levels of satisfaction with the decision-making process and in better patient compliance with therapy.

Tools for Collaborative Decision-making

To engage in high-quality decision-making, physicians should have access to information about the pros and cons

of therapy that is accurate, current, and tailored to the characteristics of their patients. Physicians also need ways to communicate that go beyond the likelihood of risk and benefit. Ideally, physicians should be able to provide patients with some sense of what the possible outcomes or health states might be like from a quality-of-life perspective.

A decision-support tool that addresses these challenges has been developed.³² The shared decision-making program (SDP) uses interactive laser disk technology to combine didactic narrative, patient testimonials, and tailored estimates of risk and benefit. The didactic information provides patients with general facts about their condition, and patient testimonials allow viewers to hear from patients who have made different choices and experienced different outcomes. The tailored presentations of risk and benefit allow the viewer to receive personalized information.

The SDP is not designed to replace the physician in the decision-making process. On the contrary, it complements the traditional physician-patient encounter. In practice, physicians identify eligible patients and advise them to view the program. An introductory brochure gives patients a working vocabulary and overview of the decision-making process. After viewing the program, the patient is encouraged to return to the physician to make a final treatment decision. SDPs are available for a broad range of clinical conditions, including benign prostatic hyperplasia^{33,34} mild hypertension, breast cancer,³⁵ low back pain, prostate cancer, prostate specific antigen testing, and others. An SDP that addresses the HRT decision is also available. The first version of the program presents risks and benefits for a 50-year-old woman with average risks for the diseases in question. A version that tailors presentations of risks and benefits according to the viewer's clinical characteristics also is being developed.

The SDP for HRT covers topics ranging from changes occurring with menopause to relief of symptoms associated with estrogen deficiency, reducing risks of heart disease and osteoporosis, and possible harms of HRT. Alternative therapies are also introduced. Viewers hear from women who have chosen to take hormones and from others who decided against hormone therapy. In addition to providing information about risks and benefits in relative terms, the program presents risks and benefits in absolute terms. For example, women first learn that long-term HRT may reduce the likelihood of developing coronary artery disease by about 50%. They then see, in graphic format, what that means to a cohort of 100 50-year-old women over the rest of their lifetimes. This is compared and contrasted with estimates of the cohort's lifetime risk of developing hip fracture, breast cancer, and endometrial cancer, with and without hormones. In ad-

dition to examining the lifetime risks of disease, women are encouraged to consider their own values and preferences and how they feel about preventing osteoporosis or heart disease, whether they would worry about breast cancer, and how they feel about costs and side effects of medications.

Use of an SDP among men with benign prostatic hyperplasia has shown that patients rate the SDP very favorably.³³ These SDP users had treatments that were consistent with their preferences and attitudes about alternative health states associated with the treatment options. Results from a randomized controlled trial of SDP for men with benign prostatic hyperplasia are forthcoming.

The SDP is only one of the available interventions. Other decision-support tools are being developed to assist in collaborative decision-making and to promote patient involvement in their health care. For example, the Comprehensive Health Enhancement Support System uses on-line computer technology to provide patients with access to general information, bulletin board discussion groups, and expert opinion about a number of health conditions. The program has been successfully piloted.^{36,37} Researchers are also developing hand-held decision-making tools to facilitate discussions of risks and benefits.³⁸ In addition, self-help and reference texts for patients³⁹ are widely available and are increasingly being employed in managed care settings.

Summary

Exploring the medical practice variation phenomenon has led to a clearer understanding of the importance of patients' preferences and of the likely benefits of involving patients more actively in their care, but more research is needed. The primary care setting is ideal for studying these issues. The preceding discussions have used the HRT decision to highlight the influence of scientific uncertainty, physicians' recommendations, and patients' preferences in making clinical decisions. A proposal has been made to invite willing patients to participate to a greater degree in their care, but it has been recommended that the impact of this process of care be closely monitored. Decision-support tools show promise for facilitating the process of collaborative decision-making.

There are six broad areas in which research is needed: (1) assessing patients' readiness for collaboration; (2) describing the nature and durability of patients' decision-making styles; (3) assessing physicians' preparedness for collaboration; (4) understanding the process of collaborative decision-making; (5) assessing the benefits and costs of collaborative decision-making; and (6) assessing

the value of technology and decision tools in the collaborative decision-making process.

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