The Impact of Expressions of Treatment Efficacy and Out-of-pocket Expenses on Patient and Physician Interest in Osteoporosis Treatment: Implications for Pay-for-performance Programs

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BACKGROUND: Clinical practice guidelines (CPGs) are increasingly used as the basis for pay-for-performance (P4P) programs. It is unclear how support for guidelines varies when treatment efficacy is expressed in varying mathematically equivalent ways.

OBJECTIVES: To assess: (1) how patient and provider compliance with osteoporosis CPGs varies when pharmacotherapy efficacy is presented as relative risk reduction (RRR) versus absolute risk reduction (ARR) and (2) the impact of increasing out-of-pocket drug expenditures on acceptance of guideline concordant therapy.

DESIGN: Cross-sectional survey of patients and physicians.

SUBJECTS AND SETTING: Female patients age >50 years and providers drawn from academic and community outpatient clinics.

MEASUREMENTS: Patient and provider acceptance of pharmacotherapy when treatment efficacy (reduction in hip fractures) was expressed alternatively in relative terms (35% RRR) versus absolute terms (1% ARR); acceptance of pharmacotherapy as patient drug copayment increased from 0% to 100% of the total drug costs.

RESULTS: Compliance with CPGs fell significantly when the expression of treatment benefit was switched from RRR to ARR for both patients (86% vs 57% compliance; $P<.001$) and physicians (97% vs 56% compliance; $P<.001$). Increasing drug copayment from 0% to 10% of total drug cost decreased patient compliance with CPGs from 80% to 57% ($P<.001$) but did not impact physician compliance. With increasing levels of copay, both patient and provider interest in treatment decreased.

LIMITATIONS: Respondents may not have fully understood the risks and benefits associated with osteoporosis and its treatment.

CONCLUSION: Patient and provider interest in CPG-recommended treatment for osteoporosis is reduced

when treatment benefit is expressed as ARR rather than RRR. In addition, minimal increases in drug copayment significantly decreased patient, but not provider, interest in osteoporosis treatment. Designers of P4P programs should consider details including expressions of treatment benefit and patients' out-of-pocket costs when developing measures to assess quality-of-care.

KEYWORDS: treatment efficacy; out-of-pocket expenses; osteoporosis treatment; pay-for-performance programs.

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INTRODUCTION

Pay-for-performance (P4P) based on adherence to clinical practice guidelines (CPGs) is becoming commonplace in clinical practice in the United States.^{1–5} As financial incentives to comply with CPGs grow, provider and patient autonomy may increasingly come into conflict with these guidelines. Treatment decisions related to osteoporosis highlight these potential conflicts.

Each year, an estimated 1.5 million Americans experience osteoporosis-related fractures, resulting in annual costs of approximately \$18 billion.^{6,7} Increasingly, CPGs advocate screening high-risk individuals (e.g., postmenopausal women age >65 years) with dual energy x-ray absorptiometry (DXA) scans to identify those with reduced bone density before sentinel fractures occur.^{8,9} Such a strategy is predicated upon the assumption that patients and providers will make informed decisions to initiate antiresorptive therapy when DXA scans demonstrate osteoporosis. Payers and specialty societies have recommended osteoporosis guideline adherence be incorporated into P4P programs.^{10,11} Whereas improving osteoporosis care is important, it is unclear whether patients and providers support the aggressive care prescribed by most guidelines. In particular, it is uncertain how enthusiasm for such aggressive osteoporosis therapy might vary depending upon the manner in which the benefits of osteoporosis treatment are presented.

Treatment benefits are most often presented in relative terms, such as relative risk reduction (RRR; Table 1).¹² Increasingly, however, health numeracy experts are recommending that treatment benefits also be presented in absolute terms, such as

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Table 1. Definitions of Absolute Risk Reduction, Relative Risk Reduction and Number Needed to Treat

Terms	Definitions
Absolute risk reduction (ARR)	The difference between the control group's event rate (CER) and the experimental group's event rate EER). $ARR = CER - EER$
Relative risk reduction (RRR)	The percentage reduction in events in the treated group compared to the control group rate. $RRR = (CER - EER) / CER$
Number needed to treat (NNT)	The number of patients that must be treated to avoid 1 adverse outcome. $NNT = 1 / ARR$

absolute risk reduction (ARR) and number needed to treat (NNT).^{13,14} For example, bisphosphonate treatment of women ages 65–69 with osteoporosis can be expected to reduce the risk of hip fracture over 5 years from approximately 3% to approximately 2%. This can be presented as a 35% RRR or a 1% ARR.⁹ These are both accurate expressions of the same treatment benefit afforded by bisphosphonates. The rationale for recommending ARR/NNT over RRR stems from the fact that empirical studies suggest that both patients and providers overestimate treatment benefits when they are only provided with RRR data.^{14,15}

We surveyed patients and physicians to examine how compliance with osteoporosis CPGs varied when treatment benefits were presented in relative (RRR) versus absolute terms (ARR). We also examined how patient and physician compliance varied when patients' out-of-pocket drug costs increased. We hypothesized that patient and provider compliance with CPGs would decrease when treatment benefits were presented as ARR rather than RRR and that patients would be more sensitive to minimal increases in drug copayment than physicians.

METHODS

Survey Development

We developed a 14-item survey (see Appendix 1) to assess patient and physician compliance with CPGs for osteoporosis screening and treatment using different clinical scenarios in which treatment benefit was presented alternatively in relative or absolute terms. The survey requested basic demographic information including age, gender, current use of osteoporosis medications (patients only), and years in practice (providers only).

The survey contained 5 separate questions that evaluated 2 domains: (1) how the presentation of treatment efficacy as RRR or ARR affected respondent compliance with CPGs as determined by their interest in osteoporosis therapy and (2) how prescription drug coverage (and patient out-of-pocket drug cost) impacted CPG compliance. The patient and physician versions of the survey were identical with the exception that patients were asked about their personal interest in osteoporosis therapy, whereas physicians were asked about recommending therapy to a hypothetical 65-year-old patient.

Estimates of treatment efficacy were primarily based upon a structured literature review on osteoporosis screening and treatment that was conducted for the U.S. Preventive Services Task Force; this review reported that bisphosphonate therapy reduced the relative risk of hip fracture in osteoporotic women 65–69 years old by 35% with an absolute reduction in hip fracture risk of 1%.¹⁶ Treatment costs for osteoporosis were based upon the cost of bisphosphonate therapy at an online pharmacy in June 2006.¹⁷

The first 2 questions assessed baseline interest in osteoporosis screening (question 1) and treatment (question 2) before respondents were given any information on treatment efficacy or cost. The third and fifth questions assessed interest in treatment when respondents were given information about treatment efficacy, expressed in relative terms (question 3) or absolute terms (question 5). The fourth question assessed the influence of prescription drug coverage on interest in osteoporosis treatment among patients and providers.

The survey was pilot tested on 15 patients to insure clarity and readability. Based upon their suggestions, only minor changes were made. The survey was then given to consecutive women over 50 years of age presenting to primary care clinics at a major academic medical center and a large community primary care practice. The survey was also given to all attending physicians at the 2 practice sites; our objective was to obtain completed surveys from 40 physicians, which would provide us with 93% power to detect a 20% difference in guideline compliance in question 3 compared with question 5.

Survey Analyses

We first assessed the proportion of patients and providers answering "yes" to each of the clinical scenarios; as mentioned above, this can be construed as "adhering" to the CPGs. We then compared the proportion of patients complying with CPGs when treatment benefit was presented in relative terms (question 3) and absolute terms (question 5) using chi-square testing; a similar comparison was made for the physician respondents. Similarly, we compared the proportion of patients and proportion of providers adhering to guidelines using analogous questions (e.g., question 3) to examine potential differences in patient and provider CPG compliance. In addition, we compared the proportion of patients who would accept and providers who would recommend pharmacologic therapy when insurance paid for an identical proportion of the total treatment cost (question 4).

Three hundred twelve patients were invited to participate, 267 completed the survey (86%). Fifty-two physicians were invited to participate, 36 (69%) completed the survey. This study was approved by the institutional review boards of the University of Iowa and Mercy Medical Center in Dubuque, Iowa. Analyses were conducted using StataSE Version 8.2 (StataCorp, College Station, TX, USA). There was no external funding for this study.

RESULTS

The characteristics of the respondents are displayed in Table 2. In our initial assessment of compliance with CPGs before the

Table 2. Characteristics of Respondents

	Patients (N=267)	Physicians (N=36)
Sex—male, number* (%)	0 (0)	27 (79)
Taking calcium supplementation, number (%)	178 (67)	n/a
Taking osteoporosis therapy, number (%)	51 (19)	n/a
Age, mean (standard deviation)	64 (10.5)	44 (7.2)
Years in practice, mean (standard deviation)	n/a*	12.8 (7.2)

n/a: not applicable

*Two respondents did not identify their gender.

Table 3. Number of Respondents Reporting that they Would Adhere with CPGs for each Question

	Patients (N=267)	Physicians (N=36)	P value
Agreement with screening DXA scan	246 (93)	33 (97)	.61
Agreement with pharmacotherapy for osteoporosis: no information on treatment efficacy provided	221 (83)	34 (100)	.09
Agreement with pharmacotherapy for osteoporosis: treatment efficacy provided as RRR	227 (86)	33 (97)	.27
Agreement with pharmacotherapy for osteoporosis: treatment efficacy provided as ARR	147 (57)	19 (56)	.64

Full text of questions available in Appendix 1.

CPG: clinical practice guideline, DXA: dual energy x-ray absorptiometry, RRR: relative risk reduction, ARR: absolute risk reduction

presentation of any data on the efficacy of bisphosphonate therapy, 93% of patients and 100% of physicians reported that they would favor screening DXA scanning; in a follow-up question, 83% of patients and 100% of physicians reported that they would be interested in prescription therapy if the DXA scan showed osteoporosis.

When information on treatment benefit was switched from RRR to ARR (Table 3), interest in pharmacotherapy declined significantly for both patients (86% with RRR to 57% with ARR; $P<.001$) and physicians (97% to 56%; $P<.001$). In comparisons of patient and providers, interest in adhering with CPGs was similar in both the RRR and ARR scenarios (Table 3).

Increasing patients' out-of-pocket expenditures (Fig. 1) from 0% to 10% of drug cost reduced patients' guideline adherence from 80% to 57% ($P<.001$) but did not have an impact on physicians' adherence (100% vs 97%; $P=.63$). Whereas patients'

and providers' compliance was similar when insurance covered 100% of drug cost (80% vs 100%; $P=.07$) patients were significantly less likely to comply with CPGs than providers ($P<.001$) for all levels of cost sharing.

DISCUSSION

The current study suggests that both patients and physicians are less interested in aggressive treatment of osteoporosis when the benefits of treatment are presented in absolute terms (i.e., ARR) rather than relative terms (i.e., RRR). To the extent that presenting treatment benefits in absolute terms is a more accurate representation of treatment benefits, our study suggests that better informed patients and providers are less likely to choose the aggressive therapy suggested by many osteoporosis guidelines. The study also demonstrates that even a minimal increase in pharmaceutical costs both significantly reduces patients' interest in pharmacotherapy and creates a potential rift between provider recommendations of therapy and patient acceptance. These findings highlight the importance of subtle details, including health numeracy and pharmaceutical benefits, as determinants of patient and provider agreement (and compliance) with CPGs.

Health numeracy, defined as the individual-level skills needed to understand and use quantitative health information, is much more than an academic issue.¹⁸ A number of prior studies have demonstrated that patients and physicians view an array of medical therapies more favorably when the benefits of these therapies are reported in relative terms rather than absolute terms because results reported in relative terms magnify treatment benefit.¹⁹⁻²¹ For example, Bobbio et al. demonstrated that physicians' agreement to prescribe a cholesterol-lowering treatment was lower when data were presented in absolute terms (37%) versus relative terms (77%). Similarly, Hux and

Figure 1. Proportion of patients and physicians opting for bisphosphonate pharmacotherapy for osteoporosis by level of out-of-pocket expenditure

Naylor found that patients were less likely to accept cholesterol-lowering treatment when treatment benefit was presented in absolute terms as opposed to relative terms.²⁰ More recently, Halvorsen et al. found that expressions of treatment benefit in terms of postponement of an adverse event lead to less treatment than expressions of treatment benefit in absolute terms.²¹

Our study expands on existing numeracy research by looking simultaneously at how patients and providers respond when the presentation of treatment benefit is switched from RRR to ARR. The consistency of our finding that both patients' and providers' enthusiasm for therapy is reduced when treatment benefits are presented in absolute terms supports prior health numeracy research.^{19,22}

Given a general consensus that treatment benefits should be presented either primarily or exclusively as ARR/NNT, it is somewhat perplexing that so many sources of health care information present treatment efficacy primarily as RRR. In the case of osteoporosis, many of the information sources that patients and providers depend on, including CPGs,¹² present treatment benefits primarily in relative terms.^{13,23}

The finding of reduced interest in treatment when benefit is presented as ARR has a number of direct implications for patient care. First, it is important to recognize that a wide array of interest groups (e.g., pharmaceutical manufacturers, advocacy groups) have an opportunity and motive to magnify the apparent effectiveness of any treatment by reporting treatment efficacy as RRR rather than ARR. To the extent that certain CPGs present treatment benefits more prominently in relative terms than absolute terms,¹² our findings suggest that both patients and providers exposed to such CPGs might choose more aggressive therapy than if treatment benefit were presented more prominently in absolute terms.

Second, in an era of P4P, the linkage of physician reimbursement to guideline compliance is dependant upon the assumption that both patients and providers agree with the treatment plan advocated by the specific guideline.²⁴ As osteoporosis screening and treatment is incorporated into P4P plans, our study suggests that measurement of CPG compliance could actually encourage aggressive osteoporosis care that more fully informed patients and providers may not support. Third, linking reimbursement to guideline adherence has the potential to create conflicts of interest between physicians and patients. A physician under financial pressure to comply with CPGs may have incentive to order tests or treatments that his or her patient might not want, particularly if the patient is fully informed of treatment benefits in both absolute and relative terms.

Our finding that patients and physicians are less likely to choose treatment as the patient's out-of-pocket medication expenses rise is consistent with prior studies.²⁰ The finding that patients are more sensitive to small increases in their out-of-pocket expenditures than physicians is not surprising, but further highlights how seemingly small changes in health care benefits may have significant impact on patient willingness to comply with treatment.

The study has certain limitations that should be considered. First, the scenarios did not comprehensively list all risks and benefits associated with either osteoporosis or bisphosphonate therapy. To the extent that physicians have a more complete understanding of these issues than patients, this has the potential to alter our findings. Nevertheless, the clinical scenarios were deliberately balanced to provide patients and providers with similar amounts of information in an effort to minimize this

possibility. It is also important to note that this study was conducted in a highly educated and ethnically homogeneous state where health numeracy among patients is likely to be higher than average. This may have reduced the observed impact of presentation of treatment benefit on medical decision making. Finally, a single ARR was portrayed to all participants regardless of their age. This ARR was that of a 65- to 69-year-old woman and would have overestimated the risk of hip fracture for a 50-year-old woman and underestimated the risk for a 75-year-old woman.

In summary, the current study suggests that patients and physicians are less interested in guideline-concordant therapy for osteoporosis when treatment benefits were presented in absolute rather than relative terms. Furthermore, as drug copayments increased, acceptance of therapy recommended by guidelines decreased, more so for patients than providers. P4P programs that directly link physician reimbursement with patient guideline compliance have the potential to create important conflicts.

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4. The cost of treatment of osteoporosis for 5 years is \$5,000. Would you be likely to take the medication if (please answer for each scenario):
 - a. your insurance covered 10% of the cost, and you paid \$4,500. Yes___No___
 - b. your insurance covered 20% of the cost, and you paid \$4,000 Yes___No___
 - c. your insurance covered 50% of the cost, and you paid \$2,500 Yes___No___
 - d. your insurance covered 90% of the cost, and you paid \$500 Yes___No___
 - e. your insurance covered 100% of the cost, and you paid nothing Yes___No___
5. Now consider that your doctor offered you a medication to reduce your chances of a hip fracture by 1%. That is, without treatment we would expect 3 of every 100 women like you to have a hip fracture over 5 years; with the medication 2 of every 100 women like you would have a hip fracture. The side effects are minimal and your insurance pays the entire cost of the medication. Would you take it? Yes___No___
6. The cost of treatment of osteoporosis for 5 years of therapy is \$5,000. Your insurance *does not pay* for medications. You have a 1 in 100 chance that taking the medication will prevent you from having a hip fracture. Would you take the medication? Yes___No___
7. Please indicate the level at which you would consider treatment to prevent a hip fracture? 1 of every ___patients treated for 5 years would personally benefit.
8. To make screening for osteoporosis a reasonable procedure, how many patients do you feel would need to be screened and treated to prevent 1 hip fracture?___
9. The costs of preventing 1 hip fracture through screening and treatment have been estimated to be approximately \$500,000. In light of these costs do you think that this screening and treatment is a reasonable intervention? Yes___No___
10. What do you think is a reasonable cost for screening and treatment to prevent 1 hip fracture? _____

Additional information:

APPENDIX 1: PATIENT VERSION OF SURVEY

1. At your annual physical exam your doctor recommends that you have a bone density test to see how strong your bones are, and to see whether you are at risk for a fracture in the future. This is a painless test, for which you do not have to undress, and which takes about 5 min. Insurance covers the cost. Would you choose to have the test done? Yes___ No___
2. The test returned showing that you have osteoporosis, or a weakening of the bones, increasing your chances of suffering a hip fracture. You are aware of the consequences of a hip fracture, as your neighbor suffered a hip fracture and was in a nursing home for several months. You have prescription drug insurance coverage. Are you interested in treatment with prescription medication? Yes___ No___
3. Your doctor has recommended treatment with a prescription medication which would reduce your risk of fracture by about 35% over 5 years of treatment. The known side effects are minimal. Your insurance pays the entire cost of the medication. Would you be likely to take it? Yes___ No___
10. Age: ___
10. Are you currently taking calcium? ___
10. Are you currently taking a prescription medication for osteoporosis prevention or treatment? ___
10. Where do you receive your medical care? ___