

## **HHS Public Access**

Med Decis Making. Author manuscript; available in PMC 2015 July 15.

Published in final edited form as:

Author manuscript

Med Decis Making. 2012; 32(5): 656-659. doi:10.1177/0272989X12458978.

## Building Better Models: If We Build Them, Will Policy Makers Use Them? Toward Integrating Modeling into Health Care Decisions

Jeanne Mandelblatt, MD, MPH, Clyde Schechter, MD, MS, David Levy, PhD, Ann Zauber, PhD, Yaojen Chang, PhD, and Ruth Etzioni, PhD

Lombardi Cancer Center, Washington, DC (JM, DL, YC); Albert Einstein School of Medicine, New York, New York (CS); Memorial Sloan Kettering Cancer Center, New York, New York (AZ); and Fred Hutchinson Cancer Center, Seattle, Washington (RE)

This landmark issue of *Medical Decision Making* summarizes important deliberations about best practices for simulation modeling.<sup>1–7</sup> This body of work represents the third major set of recommendations for modeling best practices in the past 2 decades.<sup>8,9</sup> The current project, sponsored jointly by the International Society for Pharmacoeconomics and Outcomes Research and the Society for Medical Decision Making, builds on and extends prior efforts to set standards for the conduct of modeling.<sup>10–13</sup> These new articles provide modelers with guidance on building more useful models and consumers with benchmarks to judge the quality of the models. These thoughtful guidelines should strengthen the integrity of the model development process and encourage broader use of models in decision making.

Modeling has long been embraced by the medical decision-making community and other researchers concerned with evidence-based practices and outcomes. In 2009, simulation modeling was recommended by the Institute of Medicine as a method to quantify the net impact of medical interventions.<sup>14</sup> More recently, the Patient-Centered Outcomes Research Institute has been charged with using a variety of methods, including modeling, to evaluate the comparative effectiveness of medical interventions (www.pcori.org). In addition, more complex and biologically accurate models are now possible because of computing and information technology advances.

Until recently, however, models affected few coverage and policy decisions in the United States. Models were used in setting American Cancer Society cervical cancer screening guidelines in the 1980s.<sup>15,16</sup> In the late 1980s, the US Congress' Office of Technology Assessment (OTA) commissioned models to evaluate screening for cervical and breast cancer under the Medicare program. The results were influential in the decisions to extend Medicare benefits to include Pap smears and mammography between 1988 and 1990.<sup>17,18</sup> Unfortunately, the OTA was de-funded in 1995.<sup>19</sup> Cancer modeling gained renewed support in 1999, when the Institute of Medicine released a report on the quality of care and called for greater inclusion of cancer outcomes research at the National Cancer Institute (NCI).<sup>20</sup>

Address correspondence to Jeanne Mandelblatt, MD, MPH, Lombardi Cancer Center, Washington, DC, USA; mandelbj@georgetown.edu.

Meanwhile, outside of the United States, modeling has been and continues to be used to guide policy on a routine basis.<sup>21,22</sup> In the United Kingdom, for example, cost-effectiveness results from models are required for health care coverage decisions.<sup>21</sup>

Modeling of cancer interventions was advanced in the late 1990s with the funding of NCI's Cancer Intervention and Surveillance Modeling Network (CISNET).<sup>12</sup> Over the past 15 years, CISNET has gained traction in advancing the use of modeling to inform policy and clinical practice. Several aspects of the CISNET modeling approach have contributed to its success, including use of best modeling practices, similar to those outlined in this issue; deployment of more than one model to address a specific research question; development of a template for the description of the models; and commitment to transparency and collaboration. For example, by having multiple modeling teams working together to use a common data set, CISNET has avoided some of the difficulty that can arise when single models come to widely divergent conclusions, as happened in the assessment of spiral computed tomography (CT) scanning.<sup>23–26</sup>

Policy makers have approached CISNET to apply their extant models to inform emergent debates in cancer care. The CISNET models have been used successfully to inform Centers for Medicare & Medicaid Services (CMS) reimbursement decisions about fecal immunochemical tests, stool DNA tests, and CT-colonography for colorectal cancer screening.<sup>27–29</sup> They have also been employed by the United States Preventive Services Task Force (USPSTF) to inform their colorectal cancer<sup>30</sup> and breast cancer<sup>31</sup> screening guidelines. Finally, several CISNET models were commissioned by the Healthy People 2010 initiative to determine the feasibility and impact of reaching goals for reductions in smoking prevalence<sup>32</sup> and colorectal cancer mortality.<sup>33</sup> There are also examples of models outside of CISNET successfully informing policy and/or practice, including models of selected pharmaceuticals, radiological agents, vaccines, screening for HIV infection, and human papillomavirus testing in women with human immunodeficiency virus.<sup>34–39</sup>

Even with these few success stories of models affecting recent policy decisions, there are considerable missed opportunities to use the plethora of existing high-quality models in making important health care decisions. The case of prostate cancer screening is a good example. Leading US organizations, including the USPSTF, the American Society of Clinical Oncology, the American Cancer Society, the American Urology Association, and the National Comprehensive Cancer Network, all offer different and conflicting recommendations about prostate-specific antigen screening.<sup>40</sup> This is a situation where models have not been used directly but could contribute to the ongoing debates within these professional groups by providing a formal weighting of harm v. benefit.

There have been other obstacles to moving the field of modeling science forward, especially when modeling and politics have collided. For instance, when the USPSTF issued revised breast cancer screening recommendations in November 2009, the final week of debates on the Patient Protection and Affordable Care Act, there was enormous public, political, and scientific push-back about changes in language about the age of screening initiation.<sup>41,42</sup> The CISNET models were even used by some in the radiology community to draw erroneous conclusions about the data.<sup>43,44</sup>

Mandelblatt et al.

Several other factors are likely to contribute to continued resistance to using models in decision making in the United States, including preferences for only using clinical trial evidence, a relative lack of historical modeling standards, and perceptions of models being "black boxes." There is also variability in transparency based on the model funding source that needs to be considered by end-users of the models. Federal policies mandate data sharing, whereas the privately developed models have strong intellectual property investments that can limit disclosure of their methods.<sup>45</sup> Another potential barrier to advancing the use of modeling in US health care decisions is the turbulent political climate, including recent discussions about elimination of agencies and initiatives that support modeling.<sup>46</sup>

Notwithstanding these considerable challenges, we should not lose sight of our impressive gains. We have come a long way in the evolution of modeling, and it remains a powerful method to quantify the balance of benefits, harms, and costs of candidate medical policies. High-quality models are especially salient now, when there is an urgent need to address spiraling health care costs related to the demographic pressures of an aging population and new technologies disseminating into use ahead of evidence about their impact. This milieu, coupled with the explosion of knowledge about the biological drivers of health, provides the modeling community with exciting new opportunities, including how to simulate multilevel influences on health, the impact of the "genomic revolution" on health outcomes, and linking "under-the-skin" cellular models to population models.<sup>47</sup> Past standards have facilitated broader acceptance and use of models. The accompanying standards for best practices in this issue will provide modelers with a roadmap for building even better models and policy makers with formal criteria for selecting models to inform their recommendations. It remains to be seen whether use of these best practices will be practical and, ultimately, whether they will facilitate more widespread use of high-quality models to inform future health care policies.

## References

- Caro JJ, Briggs AH, Siebert U, Kuntz KM. Modeling good research practices—overview: a report of the ISPOR-SMDM Modeling Good Research Practices Task Force–1. Med Decis Making. 2012; 32(5):667–677. [PubMed: 22990082]
- Karnon J, Stahl J, Brennan A, Caro JJ, Mar J, Möller J. Modeling using discrete event simulation: a report of the ISPOR-SMDM Modeling Good Research Practices Task Force–4. Med Decis Making. 2012; 32(5):701–711. [PubMed: 22990085]
- Siebert U, Alagoz O, Bayoumi AM, et al. State-transition modeling: a report of the ISPOR-SMDM Modeling Good Research Practices Task Force–3. Med Decis Making. 2012; 32(5):690–700. [PubMed: 22990084]
- Pitman R, Fisman D, Zaric GS, et al. Dynamic transmission modeling: a report of the ISPOR-SMDM Modeling Good Research Practices Task Force Working Group–5. Med Decis Making. 2012; 32(5):712–721. [PubMed: 22990086]
- Roberts M, Russell LB, Paltiel AD, Chambers M, McEwan P, Krahn M. conceptualizing a model: a report of the ISPOR-SMDM Modeling Good Research Practices Task Force–2. Med Decis Making. 2012; 32(5):678–689. [PubMed: 22990083]
- Briggs AH, Weinstein MC, Fenwick EAL, Karnon J, Sculpher MJ, Paltiel AD. Model parameter estimation and uncertainty: a report of the ISPOR-SMDM Modeling Good Research Practices Task Force Working Group–6. Med Decis Making. 2012; 32(5):722–732. [PubMed: 22990087]

- Gold, MR.; Siegel, JE.; Russell, LB.; Weinstein, MC., editors. Cost-effectiveness in Health and Medicine. New York: Oxford University Press; 1996.
- Weinstein MC, O'Brien B, Hornberger J, et al. Principles of good practice for decision analytic modeling in health-care evaluation: report of the ISPOR Task Force on Good Research Practices— Modeling Studies. Value Health. 2003; 6(1):9–17. [PubMed: 12535234]
- Saha S, Hoerger T, Pignone M, Teutsch S, Helfand M, Mandelblatt J. The art and science of incorporating cost effectiveness into evidence-based recommendations for clinical preventive services. Am J Prev Med. 2001; 20(3, Suppl):36–43. [PubMed: 11306230]
- Neumann PJ, Johannesson M. From principle to public policy: using cost-effectiveness analysis. Health Aff (Millwood). 1994; 13(3):206–14. [PubMed: 7927151]
- 12. Cancer Intervention and Surveillance Modeling Network. [Accessed 20 July 2012] Modeling to guide public health research and priorities. Available from: http://cisnet.cancer.gov/
- Ramsey S, Willke R, Briggs A, et al. Good research practices for cost-effectiveness analysis alongside clinical trials: the ISPOR RCT-CEA Task Force report. Value Health. 2005; 8(5):521– 33. [PubMed: 16176491]
- Institute of Medicine. Initial National Priorities for Comparative Effectiveness Research. Board on Health Care Services; Available from: http://www.iom.edu/en/reports/2009/ comparativeeffectivenessresearchpriorities.aspx [Accessed 1 July 2012]
- Eddy D. ACS report on the cancer-related health checkup. CA Cancer J Clin. 1980; 30(4):193–240. [PubMed: 6774802]
- Eddy DM. The frequency of cervical cancer screening: comparison of a mathematical model with empirical data. Cancer. 1987; 60(5):1117–22. [PubMed: 3607728]
- Muller, CM.; Mandelblatt, J.; Schechter, C. The Cost and Effectiveness of Screening for Cervical Cancer in Elderly Women: A Paper in the OTA's Series on Preventive Services under Medicare. Washington, DC: US Congress, Health Program, Office of Technology Assessment; 1990.
- Eddy, D. Breast Cancer Screening for Medicare Beneficiaries: Effectiveness, Costs to Medicare and Medical Resources Required. Washington, DC: U.S. Congress, Health Program, Office of Technology Assessment; 1987.
- 19. The OTA Legacy. [Accessed 1 July 2012] Available from: http://www.princeton.edu/~ota/
- 20. National Cancer Policy Board, Institute of Medicine and National Research Council. Ensuring Quality Cancer Care. Washington, DC: National Academies Press; 1999.
- 21. National Institute for Health and Clinical Excellence. [Accessed 24 July 2012] Available from: www.nice.org.uk/
- 22. Elsinga E, Rutten FF. Economic evaluation in support of national health policy: the case of the Netherlands. Soc Sci Med. 1997; 45(4):605–20. [PubMed: 9226785]
- Wisnivesky JP, Mushlin AI, Sicherman N, Henschke C. The cost-effectiveness of low-dose CT screening for lung cancer: preliminary results of baseline screening. Chest. 2003; 124(2):614–21. [PubMed: 12907551]
- Manser R, Dalton A, Carter R, Byrnes G, Elwood M, Campbell DA. Cost-effectiveness analysis of screening for lung cancer with low dose spiral CT (computed tomography) in the Australian setting. Lung Cancer. 2005; 48(2):171–85. [PubMed: 15829317]
- Marshall D, Simpson KN, Earle CC, Chu C. Potential cost-effectiveness of one-time screening for lung cancer (LC) in a high risk cohort. Lung Cancer. 2001; 32(3):227–36. [PubMed: 11390004]
- Mahadevia PJ, Fleisher LA, Frick KD, Eng J, Goodman SN, Powe NR. Lung cancer screening with helical computed tomography in older adult smokers: a decision and cost-effectiveness analysis. JAMA. 2003; 289(3):313–22. [PubMed: 12525232]
- 27. Van Ballegooijen, M.; Habbema, JDF.; Boer, R.; Zauber, A.; Brown, ML. Report to the Agency for Healthcare Research and Quality: a comparison of the cost-effectiveness of fecal occult blood tests with different test characteristics in the context of annual screening in the Medicare population. Aug. 2003 Accessed from: http://www.cms.gov/mcd/viewtechassess.asp? where=search&tid=20

Mandelblatt et al.

- Lansdorp-Vogelaar I, Kuntz KM, Knudsen AB, Wilschut JA, Zauber AG, van BM. Stool DNA testing to screen for colorectal cancer in the Medicare population: a cost-effectiveness analysis. Ann Intern Med. 2010; 153(6):368–77. [PubMed: 20855801]
- Knudsen AB, Lansdorp-Vogelaar I, Rutter CM, et al. Cost-effectiveness of computed tomographic colonography screening for colorectal cancer in the Medicare population. J Natl Cancer Inst. 2010; 102(16):1238–52. [PubMed: 20664028]
- Zauber AG, Lansdorp-Vogelaar I, Knudsen AB, Wilschut J, van BM, Kuntz KM. Evaluating test strategies for colorectal cancer screening: a decision analysis for the U.S. Preventive Services Task Force. Ann Intern Med. 2008; 149(9):659–69. [PubMed: 18838717]
- Mandelblatt JS, Cronin KA, Bailey S, et al. Effects of mammography screening under different screening schedules: model estimates of potential benefits and harms. Ann Intern Med. 2009; 151(10):738–47. [PubMed: 19920274]
- Levy DT, Mabry PL, Graham AL, Orleans CT, Abrams DB. Reaching Healthy People 2010 by 2013: a SimSmoke simulation. Am J Prev Med. 2010; 38(3, Suppl):S373–81. [PubMed: 20176310]
- 33. Vogelaar I, Van Ballegooijen M, Schrag D, et al. How much can current interventions reduce colorectal cancer mortality in the U.S.? Mortality projections for scenarios of risk-factor modification, screening, and treatment. Cancer. 2006; 107(7):1624–33. [PubMed: 16933324]
- Neumann PJ, Claxton K, Weinstein MC. The FDA's regulation of health economic information. Health Aff (Millwood). 2000; 19(5):129–37. [PubMed: 10992660]
- 35. Eddy DM. Clinical decision making: from theory to practice: applying cost-effectiveness analysis. The inside story. JAMA. 1992; 268(18):2575–82. [PubMed: 1404827]
- Goldie SJ. Public health policy and cost-effectiveness analysis. J Natl Cancer Inst Monogr. 2003; (31):102–10. [PubMed: 12807953]
- Branson B. Current HIV epidemiology and revised recommendations for HIV testing in healthcare settings. J Med Virol. 2007; 79(Suppl 1):S6–10. [PubMed: 17874432]
- Walensky RP, Weinstein MC, Kimmel AD, et al. Routine human immunodeficiency virus testing: an economic evaluation of current guidelines. Am J Med. 2005; 118(3):292–300. [PubMed: 15745728]
- Saslow D, Castle PE, Cox JT, et al. American Cancer Society Guideline for human papillomavirus (HPV) vaccine use to prevent cervical cancer and its precursors. CA Cancer J Clin. 2007; 57(1):7– 28. [PubMed: 17237032]
- 40. Fallout over PSA guidelines continues. Cancer Lett. 2012; 38(29):1-8.
- When evidence collides with anecdote, politics, and emotion: breast cancer screening. Ann Intern Med. 2010; 152(8):531–2. [PubMed: 20157099]
- 42. Berlin L, Hall FM. More mammography muddle: emotions, politics, science, costs, and polarization. Radiology. 2010; 255(2):311–6. [PubMed: 20413746]
- Hendrick RE, Helvie MA. United States Preventive Services Task Force screening mammography recommendations: science ignored. AJR Am J Roentgenol. 2011; 196(2):W112–6. [PubMed: 21257850]
- 44. Mandelblatt J, Cronin K. Response to Hendrick and Helvie by the Cancer Intervention Surveillance Modeling Network (CISNET) Breast Working Group. AJR Am J Roentgenol. 2011; 197:W792. [PubMed: 21940561]
- 45. Schlessinger L, Eddy DM. Archimedes: a new model for simulating health care systems—the mathematical formulation. J Biomed Inform. 2002; 35(1):37–50. [PubMed: 12415725]
- 46. [Accessed 20 July 2012] 112th US Congress, 2nd session, FY13 budget, House Committee on Appropriations for the Departments of Labor, Health and Human Services, and Education, and related agencies for the fiscal year ending. Sep 30. 2013 Available from: http:// appropriations.house.gov/uploadedfiles/bills-112hr-sc-ap-fy13-laborhhsed.pdf
- 47. Morrisey J, Hassmiller-Lich K, Anhang-Price R, Mandelblatt J. Computational modeling and multilevel cancer interventions. J Natl Cancer Inst Monogr. 2012; 44:55–66.