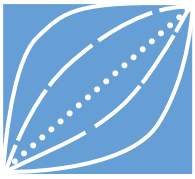
Decision Making in Health and Medicine

Integrating Evidence and Values

Second Edition



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In memoriam
Howard S. Frazier
Jane C. Weeks

Contents

Foreword

Suppose we are sitting in a room, and I tell you that if you walk out a certain door, you will die instantly. However, if you remain in your chair for another five minutes, you can leave when you like with no ill effects. If you believe me, and you value your life, you will stay put, at least for five minutes.

This situation poses an easy choice. It requires little thought and no analysis, and the best option is transparently clear. A number of attributes make this an easy case: First, the choice is stark, with only two extreme outcomes, live or die. Second, the consequences are certain-live if you stay, die if you leave. Third, the outcomes are immediate, with no time delay. Fourth, there are no financial costs involved, and if anything the preferred choice (staying in your chair) is easier than getting up and leaving. And finally, you are making this choice for yourself; you are the one who decides and who will experience the outcomes.

Unfortunately, real-life situations related to medicine and health are murkier and more complicated. The choices are much more varied than "stay or go" and may involve a range of possible tests and treatments, as well as watchful waiting. The attainable outcomes include many possible states of ill-health, ranging from minor inconvenience to severe pain and disability, as well as death. The intermediate and ultimate results are rife with uncertainty, and the various states of illness may play out over a long time period. Typically, both a doctor and a patient are involved in decision-making, and, in some cases, perhaps family members and others as well. The doctor generally knows more about what may happen, and the patient understands more about their own preferences, and both information and values bear on the best decision. Layer on top of all this the emotionality and urgency that occasionally attends to health and medical care. And then try to contemplate questions of cost and choices that can affect the health of an entire population.

Foreword

This book presents a systematic approach to identifying, organizing, and considering these many complexities in health and medical care. In a sense, all of the tools described in these pages are designed to convert the complex and uncertain realities of health and medicine into the decision-equivalent of the simple "stay or go" question posed above. For example, probability analysis converts the array of uncertain, intermediate, and ultimate states into an overall expectation of what will or will not happen (as if they were simply certain to live or certain to die). Utility assessment allows a decision maker to rank the value of all possible outcomes on a scale (between life and death) that is quantitatively meaningful for decisions. Discounting allows one to set equivalents for outcomes that play out at different times (as if the outcomes were all to occur now). Modeling enables one to take account of complex interactions and the iterative quality of many disease processes over time (as if they were clear-cut and instantaneous). Tools such as balance sheets and decision diagrams enable one to take account of the problem as a whole and to focus at different times on specific parts without losing sight of the whole, something the unaided human mind cannot possibly accomplish. And cost-effectiveness analysis enables one to consider systematically the most efficient means of achieving one's aims, when costs matter. And today, costs frequently matter.

The Institute of Medicine (IOM) has long been a champion of patient-centered care. It is one of the six core attributes of quality defined by the IOM and arguably the most fundamental aim. If we are truly centered on the needs of patients, and on the health needs of people more generally, the goals of safe, effective, timely, efficient, and equitable care naturally follow as part of attaining higher-quality care. The tools and techniques outlined in this text will not make an uncaring physician more compassionate, nor an indifferent caregiver more centered on the needs of the patient. However, for doctors who are compassionate and caring, these tools will strengthen their ability to reach decisions with patients that truly serve the patient's interests in health and medicine.

Harvey V. Fineberg, M.D., Ph.D.
President, Institute of Medicine
June 2014

Foreword to the first edition (2001)

. . . high Arbiter *Chance* governs all.

John Milton, *Paradise Lost*, book II, lines 909–10

When the predecessor to this book was being prepared in the late 1970s (Weinstein et al., 1980), medical decision making seemed to have become more complicated than ever before. The number of diagnostic and therapeutic options dwarfed those of an earlier generation, and the costs of care were growing relentlessly. Increasing numbers of patients expected to play an active role in decisions that affected their lives, and many physicians were acclimating themselves to a less authoritarian doctor–patient relationship. The tools of decision analysis permitted the clinician and patient to break down the complexity of a medical situation into its constituent parts, to identify and assess the pertinent uncertainties and values, and to reassemble the pieces into a logical guide to action.

Today, a generation later, the dilemma of medical decision making seems even more problematic. This is not merely the result of scientific and technologic advances – ingenious new devices, pharmaceuticals, surgical possibilities, and other interventions. The environment of decision making has itself become confounded by government agencies and service delivery systems playing a more direct (and directive) role in decision making. Today, not only are the costs of care a prime concern, so, too, is the quality of care. Patients no longer need rely mainly on their physicians to gain access to medical information – the internet has given millions a direct line to abundant information, though of variable accuracy and pertinence. In light of progress in mapping the human genome, clinicians may soon face profound ethical questions that only a generation ago were the stuff of science fiction.

These dynamic changes in medicine, in science, and in the health-care environment make this new book more valuable than ever. This volume

conveys both fundamental and sophisticated methods that can render complex health-care situations more comprehensible. It would be a mistake, however, to think that the methods described in this volume apply only to the exceptional case, to the rare clinical encounter. The task of integrating scientific knowledge, clinical evidence, and value judgments into coherent decisions remains the daily task of medical care.

Much of what counts for differences in outcome related to medicine comes not from failure to access experimental and expensive technology. It comes rather from the failure to deploy relatively inexpensive and proven technology to all those who need it: vaccine against pneumonia for those at risk, beta-blockers in the period following myocardial infarction, appropriate screening for cancer, and much more. The challenge for quality improvement is not the extraordinary case and exceptional decision so much as the challenge to implement systematically the preventive, diagnostic, and therapeutic measures for all who would benefit at reasonable cost. The lessons in this book can reinforce the case for sounder everyday decisions in medicine and health care.

Regardless of how far science and health care advance, the element of chance will remain a fixture in medical encounters. A refined understanding of causation and prognosis will alter how much we know about the likelihood of certain consequences, but uncertainty will persist. Much of medical learning can be interpreted as an effort to reduce the range of uncertainty in medical care. The ideas and methods provided in this volume teach how to make informed decisions in the face of the uncertainty which inevitably remains.

The methods in this book to aid decision makers are simply tools. They are tools for the willing clinician. They are tools for the worried patient. They are tools for the concerned policy maker and payer. They will not make a hazardous situation safe, nor will they make a lazy or incompetent clinician into a superior caregiver. If the methods do not eliminate controversy, they can clarify the reasons for differences of opinion. In dealing with the realities and uncertainties of life and illness, they will enable the thoughtful clinician, the honest patient, and the open-minded policy maker to reach more reasoned conclusions.

Provost, Harvard University **Harvey V. Fineberg**

REFERENCE

Weinstein MC, Fineberg HV, Elstein AS, et al. *Clinical Decision Analysis*. Philadelphia, USA: WB Saunders; 1980. ISBN 0-7216-9166-8.

Preface

How often do you find yourself struggling with a decision, be it a medical decision, a policy decision, or a personal one? In clinical medicine and health-care policy, making decisions has become a very complicated process: we have to make trade-offs between risks, benefits, costs, and preferences. We have to take into account the rapidly increasing evidence – some good, some poor – presented in scientific publications, on the worldwide web, and by the media. We have to integrate the best available evidence with the values relevant to patient and society; and we have to reconcile our intuitive notions with rational analysis.

In this book we explain and illustrate tools for integrating quantitative evidence-based data and subjective outcome values in making clinical and health-policy decisions. The book is intended for all those involved in clinical medicine or health-care policy who would like to apply the concepts from decision analysis to improve their decision making process. The audience we have in mind includes (post-)graduate students and health-care professionals interested in medical decision making, clinical decision analysis, clinical epidemiology, evidence-based medicine, technology assessment in health care, and health-care policy. The main part of the book is written with graduate students as audience in mind. Some chapters cover advanced material and as such we would recommend reserving this material for advanced courses in decision modeling (the second half of Chapters 4 and 7, and the entire Chapters 10, 11, and 12).

The authors' backgrounds ensure that this is a multidisciplinary text. Together we represent general practice, internal medicine, radiology, mathematics, decision sciences, psychology of decision making, health economics, health-care policy and management. The examples in the book are taken from both clinical practice and from health policy.

There is a previous version of this book (Weinstein et al., 1980), but the name of the book has changed, the content is 80% different, the publisher has changed, and the list of authors has changed. The main message is, however, the same: decisions in clinical medicine and health care in general can benefit from a proactive approach to decision making in which evidence and values are integrated into one framework. In addition, substantial changes have been made compared with the first edition of this book (Hunink et al., 2001): Chapters 11 and 13 are totally new, all existing chapters have been thoroughly revised to present current insights, examples throughout the book have been updated to be clinically relevant in today's practice, figures have been improved (especially in Chapter 6) and more figures have been added, and the supplementary material has been expanded and revised.

The book comes with a website. The book itself can, however, be read without immediate access to the website, that is, in a comfortable chair or on a couch! The website supplies additional materials: assignments and their solutions, examples of the decision models in the book programmed using decision analytical software, supplementary materials for the chapters including some useful spreadsheets and model templates, and the references. Access to the teachers' website, which contains additional useful material, is available on request.

We hope you enjoy reading. Good (but calculated) luck with your decision making!

M.G. Myriam Hunink
on behalf of all the authors

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Hunink MGM, Glasziou PP, Siegel JE, Weeks JC, Pliskin JS, Elstein AS, Weinstein MC. *Decision Making in Health and Medicine: Integrating Evidence and Values*. Cambridge: Cambridge University Press, Cambridge; 2001. ISBN 978-0521770293.

Weinstein MC, Fineberg HV, Elstein AS, et al. *Clinical Decision Analysis*. Philadelphia, USA: WB Saunders; 1980. ISBN 0-7216-9166-8.

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We would especially like to acknowledge the contributions of the authors of the previous version and the authors of the first edition of the book who were not directly involved this time: Harvey V. Fineberg, Howard S. Frazier, Duncan Neuhauser, Raymond R. Neutra, Barbara J. McNeil, Joanna E. Siegel, Jane C. Weeks, and Arthur S. Elstein. Also, we would like to thank the reviewers of the first edition.

Writing a book consists not only of putting text on paper, making illustrations, and having the chapters proofread, but also the thoughts, ideas, and intellectual input from many, too numerous to list and often difficult to identify, have played a role in getting this book together. We are grateful for the intellectual input from our colleagues, students, and postgraduates at the University of Queensland, Bond University, Ben Gurion University, University of York, Erasmus University Medical Center Rotterdam, Netherlands Institute of Health Sciences, Tufts University Medical School, Harvard School of Public Health, and members of the Society for Medical Decision Making.

Last, but certainly not least, we would like to thank our families for being supportive and giving us the opportunity to spend time working on the book during many evenings and weekends.

Abbreviations

ACP	American College of Physicians
ASR	age–sex–race
CABG	coronary artery bypass grafting
CAD	coronary artery disease
CDC	Centers for Disease Control and Prevention
CEA	carotid endarterectomy
CEA	cost-effectiveness analysis
CE25	certainty equivalent 25
CE50	certainty equivalent 50
CE ratio	cost-effectiveness ratio
CI	confidence interval
CPI	Consumer Price Index
CRC	colorectal cancer
CT	computed tomography
CTA	computed tomography angiography
CVD	cardiovascular disease
DALY	disability-adjusted life year
DRG	diagnostic-related group
DVT	deep venous thrombosis
EBCT	electron beam computed tomography
EKG	electrocardiogram
EQ-5D	EuroQol with five dimensions
EU	expected utility
EVCI	expected value of clinical information
EVPI	expected value of perfect information
EVPPI	expected value of partial perfect information
EVSI	expected value of sample information
FNR	false-negative ratio
FOBT	fecal occult blood test

List of abbreviations

FPR	false-positive ratio
HBV	hepatitis B virus
HDL	high-density lipoprotein
HIV	human immunodeficiency virus
HMO	health maintenance organization
HRR	hazard rate ratio
HUI	Health Utilities Index
IV	intravenous
LE	life expectancy
LR	likelihood ratio
MeSH	Medical Subject Headings
MI	myocardial infarction ('heart attack')
MISCAN	Microsimulation of Screening for Cancer
MRI	magnetic resonance imaging
MRA	magnetic resonance angiography
NHB	net health benefit
NMB	net monetary benefit
OME	otitis media with effusions ('glue ear')
OR	odds ratio
ORS	oral rehydration solution
PAD	peripheral artery disease
PAT	paroxysmal atrial tachycardia
PE	pulmonary embolism
PTA	percutaneous transluminal angiography
PV	present value
QALE	quality-adjusted life expectancy
QALY	quality-adjusted life year
QWB	Quality of Well-Being scale
RCT	randomized controlled trial
ROC	receiver operating characteristic
RR	relative risk
RRR	relative risk reduction
RRTO	risk–risk trade-off
RS	rating scale
SF-36	36-Item Short Form
SG	standard gamble
SIP	Sickness Impact Profile
TNR	true-negative ratio
TPR	true-positive ratio
VAS	visual analog scale
V/Q scan	ventilation–perfusion scan
WTP	willingness to pay

About the authors

M.G. Myriam Hunink, B.Sc., M.D., Ph.D. trained and practiced as an interventional and cardiovascular radiologist. Currently she directs the Assessment of Radiological Technology (ART) program and the division of Clinical Epidemiology at the Erasmus MC and dedicates herself to research and teaching. She is Professor of Clinical Epidemiology and Radiology at the Erasmus University Medical Center, Rotterdam, the Netherlands and Adjunct Professor of Health Decision Sciences at Harvard School of Public Health, Harvard University, Boston. She is a past president of the Society for Medical Decision Making and a recipient of their Distinguished Service award. Her main research interests are comparative effectiveness research and health technology assessment studies of diagnostic and prognostic imaging tests (biomarkers) and image-guided therapies, in particular for cardiovascular disease. Other research interests include integrated diagnostics, computerized decision support for evidence-based use of imaging tests, and (imaging to measure) the effectiveness of lifestyle interventions. Her vision is to optimize medical decisions by combining the best available quantitative evidence on risks and benefits from diverse sources and integrating patient values, preferences, quality of life, and costs.

Milton C. Weinstein, A.B./A.M., M.P.P., Ph.D. is the Henry J. Kaiser Professor of Health Policy and Management at the Harvard School of Public Health and Professor of Medicine at the Harvard Medical School. At the Harvard School of Public Health he is Academic Director of the Center for Health Decision Science, and Director of the Program on Economic Evaluation of Medical Technology. He is best known for his research on cost-effectiveness of medical practices and for developing methods of economic evaluation and decision analysis in health care. He is a co-developer of the CEPAC (Cost-Effectiveness of Preventing AIDS Complications) computer

simulation model, and has conducted studies on prevention and treatment of HIV infections. He was the co-developer of the Coronary Heart Disease Policy Model, which has been used to evaluate the cost-effectiveness of cardiovascular prevention and treatment. He consults with industry and government and is a Principal Consultant with Optuminsight. He is an elected member of the Institute of Medicine of the National Academy of Sciences, a past president and recipient of the Career Achievement Award of the Society for Medical Decision Making, and the Avedis Donabedian Lifetime Achievement Award from the International Society for Pharmacoeconomics and Outcomes Research.

Eve Wittenberg, M.P.P., Ph.D. is a Senior Research Scientist at the Center for Health Decision Science at the Harvard School of Public Health. Her interests include the conceptualization and measurement of well-being and values across individuals and health conditions, and the application of this information to clinical decision making and policy. She has estimated economic utilities for conditions ranging from cancer to intimate partner violence, and has a special focus on health issues for vulnerable and under-studied populations. Her recent work includes studies of choice-based methods to inform preference research and design of interventions, as well as measurement approaches to capture family spillover effects of illness. She teaches in the decision science curriculum at the Harvard School of Public Health and advises students in health policy.

Michael F. Drummond, B.Sc., M.Com., D.Phil. is Professor of Health Economics and former Director of the Centre for Health Economics at the University of York, UK. His particular field of interest is in the economic evaluation of health care treatments and programmes. He has undertaken evaluations in a wide range of medical fields including care of the elderly, neonatal intensive care, immunization programs, services for people with AIDS, eye health care and pharmaceuticals. He has acted as a consultant to the World Health Organization and was Project Leader of a European Union Project on the Methodology of Economic Appraisal of Health Technology. He has been President of the International Society of Technology Assessment in Health Care, and the International Society for Pharmacoeconomics and Outcomes Research. He was previously a member of the Guidelines Review Panels of the National Institute for Health and Clinical Excellence (NICE) in the UK, is a Principal Consultant for Optuminsight, and editor-in-chief of Value in Health. He has been awarded two honorary degrees, from City University, London (2008) and Erasmus University, Rotterdam (2012). In 2010 he was made a member of the Institute of Medicine of the National Academies in the USA and in 2012 he was the recipient of The John Eisenberg Award, in

recognition of exemplary leadership in the practical application of medical decision-making research, by the Society for Medical Decision Making.

Joseph S. Pliskin, B.Sc., S.M., Ph.D. is the Sidney Liswood Professor of Health Care Management at Ben Gurion University of the Negev, Beer-Sheva, Israel. He was chairman of the Department of Health Systems Management and is a member of the Department of Industrial Engineering and Management. He is also an Adjunct Professor in the Department of Health Policy and Management at the Harvard School of Public Health, Boston, USA. His research interests focus on clinical decision making, operations management in health care organizations, cost–benefit and cost-effectiveness analysis in health and medicine, technology assessment, utility theory, and decision analysis. He has published extensively on issues relating to end-stage renal disease, heart disease, Down syndrome, technology assessment, and methodological issues in decision analysis. In 2004 he received the Career Achievement Award of the Society for Medical Decision Making and in 2012 he was the recipient of a Harvard School of Public Health teaching award.

John B. Wong, B.S., M.D. is a general internist, Chief of the Division of Clinical Decision Making at Tufts Medical Center, Director of Comparative Effectiveness Research at Tufts Clinical Translational Science Institute, and Professor of Medicine at the Tufts University School of Medicine. He is a past president of the Society for Medical Decision Making and a recipient of their Distinguished Service award. He has been an invited member of the ISPOR-SMDM Modeling Good Research Practices Task Force and of guideline committees for the American Association for the Study of Liver Disease, European League Against Rheumatism, the AMA Physician Consortium for Performance Improvement Work Groups on Coronary Artery Disease, Hypertension, Heart Failure, Cardiac Imaging, and Hepatitis C, and the Technical Panel for the ACCF Appropriate Use Criteria for Diagnostic Catheterization and Multi-modality Imaging. His research focuses on the application of decision analysis to help patients, physicians, and policymakers choose among alternative tests, treatments, or policies, thereby promoting rational evidence-based efficient and effective patient-centered care. As a content editor at the Informed Medical Decisions Foundation, he has helped develop award winning decision aid programs for testing, treatment, and management of heart disease to facilitate shared decision making.

Paul P. Glasziou, F.R.A.C.G.P., Ph.D. is Professor of Evidence-based Medicine and Director, Centre for Research in Evidence-based Practice, Bond University, Australia. He was a general practitioner at the Inala Community Health Centre, and at Beaumont St, Oxford. He teaches evidence-based

practice to medical students and other health care workers. He holds honorary positions as Professor at the University of Oxford, and Professor at the University of Sydney. Dr Glasziou was the co-editor of the BMJ's *Journal of Evidence-Based Medicine*, and Director of the Centre for Evidence-based Medicine, University of Oxford. His research interests focus on identifying and removing the barriers to using high-quality research in everyday clinical practice.