

2742-4009

Quality of Life and Pharmacoeconomics in Clinical Trials

Second Edition

Editor

Bert Spilker, Ph.D., M.D.

President

Orphan Medical, Inc.

Minnetonka, Minnesota

Adjunct Professor of Medicine

Adjunct Professor of Pharmacology

Clinical Professor of Pharmacy

University of North Carolina Schools of Medicine and Pharmacy

Chapel Hill, North Carolina

Clinical Professor of Pharmacy Practice

University of Minnesota School of Pharmacy

Minneapolis, Minnesota



Lippincott - Raven

P U B L I S H E R S

Philadelphia • New York

Contents

Contributors.....	xv
Preface to the First Edition	xxxii
Preface to the Second Edition.....	xxxiii
Acknowledgments for the First Edition.....	xxxv
Acknowledgments for the Second Edition	xxxvii
About the Editor	xxxix
Other Books by Bert Spilker.....	xli
Selected List of Abbreviations Used in the Text.....	xlili

I: Introduction to the Field of Quality of Life Trials

1. Introduction.....	1
<i>Bert Spilker</i>	
2. Quality of Life Studies: Definitions and Conceptual Issues.....	11
<i>Harvey Schipper, Jennifer J. Clinch, and Charles L. M. Olweny</i>	
3. Taxonomy of Quality of Life.....	25
<i>Bert Spilker and Dennis A. Revicki</i>	
4. The Hierarchy of Patient Outcomes.....	33
<i>James F. Fries and Gurkirpal Singh</i>	
5. Measurements in Clinical Trials: Choosing the Right Approach.....	41
<i>Gordon H. Guyatt, Roman Jaeschke, David H. Feeny, and Donald L. Patrick</i>	
6. How to Develop and Validate a New Health-Related Quality of Life Instrument	49
<i>Elizabeth F. Juniper, Gordon H. Guyatt, and Roman Jaeschke</i>	
7. Adopting Higher Standards for Quality of Life Trials	57
<i>Bert Spilker</i>	
8. On Learning and Understanding Quality of Life	59
<i>Susan C. Hedrick, Richard C. Taeuber, and Pennifer Erickson</i>	
9. Normalcy, Supernormalcy, and Subnormalcy	65
<i>Bert Spilker</i>	
10. Future Directions of Quality of Life Research.....	73
<i>Paul D. Cleary</i>	

II: Standard Scales, Tests, and Approaches to Quality of Life Assessments

11.	Economic Scales and Tests	79
	<i>Henry G. Grabowski and Ronald W. Hansen</i>	
12.	Integrating Economic Evaluations and Quality of Life Assessments	85
	<i>David H. Feeny, George W. Torrance, and Roberta Labelle</i>	
13.	Social Interaction Tests and Scales.....	97
	<i>Edward Guadagnoli, Paul Nordberg, and Vincent Mor</i>	
14.	Cognitive Aspects of Quality of Life Assessment.....	107
	<i>Ivan Barofsky</i>	
15.	Psychological Aspects of Health-Related Quality of Life Measurement: Tests and Scales.....	117
	<i>Michelle J. Naughton, Sally A. Shumaker, Roger T. Anderson, and Susan M. Czajkowski</i>	
16.	Functional Disability Scales.....	133
	<i>William D. Spector</i>	
17.	Instruments to Measure Spiritual Status	145
	<i>Jan M. Ellerhorst-Ryan</i>	
18.	Pharmacoeconomics and Quality of Life Research Beyond the Randomized Clinical Trial	155
	<i>Ron D. Hays, Cathy D. Sherbourne, and Samuel A. Bozzette</i>	

III: Specific Scales, Tests, and Measures

19.	Dartmouth COOP Functional Health Assessment Charts: Brief Measures for Clinical Practice.....	161
	<i>Eugene C. Nelson, John H. Wasson, Deborah J. Johnson, and Ron D. Hays</i>	
20.	Derogatis Affects Balance Scale: DABS.....	169
	<i>Leonard R. Derogatis and Peter J. Rutigliano</i>	
21.	The European Organization for Research and Treatment of Cancer (EORTC) Modular Approach to Quality of Life Assessment in Oncology: An Update	179
	<i>Neil K. Aaronson, Ann M. Cull, Stein Kaasa, and Mirjam A. G. Sprangers</i>	
22.	The EuroQoL Instrument: An Index of Health-Related Quality of Life	191
	<i>Paul Kind</i>	
23.	The Functional Assessment of Cancer Therapy (FACT) and Functional Assessment of HIV Infection (FAHI) Quality of Life Measurement System.....	203
	<i>David F. Cella and Amy E. Bonomi</i>	
24.	The Functional Living Index—Cancer: Ten Years Later	215
	<i>Jennifer J. Clinch</i>	
25.	The Health Assessment Questionnaire 1995—Status and Review.....	227
	<i>Dena R. Ramey, James F. Fries, and Gurkirpal Singh</i>	
26.	Health Utilities Index	239
	<i>David H. Feeny, George W. Torrance, and William J. Furlong</i>	

27.	Measuring Health State Preferences and Utilities: Rating Scale, Time Trade-Off, and Standard Gamble Techniques.....	253
	<i>Kathryn J. Bennett and George W. Torrance</i>	
28.	The McMaster Health Index Questionnaire.....	267
	<i>Larry W. Chambers</i>	
29.	Nottingham Health Profile	281
	<i>James McEwen and Stephen P. McKenna</i>	
30.	Psychological Adjustment to Illness Scale: PAIS and PAIS-SR.....	287
	<i>Leonard R. Derogatis and Megan P. Fleming</i>	
31.	Quality of Life—Cancer.....	301
	<i>Geraldine V. Padilla, Marcia M. Grant, Betty R. Ferrell, and Cary A. Presant</i>	
32.	The General Health Policy Model: An Integrated Approach	309
	<i>Robert M. Kaplan and John P. Anderson</i>	
33.	SCL-90-R and the BSI	323
	<i>Leonard R. Derogatis and Maureen F. Derogatis</i>	
34.	The SF-36 Health Survey.....	337
	<i>John E. Ware, Jr.</i>	
35.	The Sickness Impact Profile.....	347
	<i>Anne M. Damiano</i>	
36.	The World Health Organization Quality of Life (WHOQOL) Assessment Instrument	355
	<i>Silvija Szabo (on behalf of the WHOQOL Group)</i>	

IV. Choosing and Administering Tests and Treatments

37.	Using Quality of Life Tests for Patient Diagnosis or Screening, or to Evaluate Treatment	363
	<i>Lisa V. Rubenstein</i>	
38.	Choosing a Health Profile (Descriptive) and/or a Patient-Preference (Utility) Measure for a Clinical Trial.....	375
	<i>Richard A. Berzon, Josephine A. Mauskopf, and George P. Simeon</i>	
39.	Data Collection Methods.....	381
	<i>Floyd J. Fowler, Jr.</i>	
40.	Narrative and Quality of Life.....	387
	<i>Deborah R. Gordon and Eugenio Paci</i>	
41.	Group Dynamics and Focus Groups.....	397
	<i>Richard A. Krueger</i>	
42.	Incorporating Trade-Offs in Quality of Life Assessment	403
	<i>Andrew J. Martin, Thomas M. Lumley, and R. John Simes</i>	
43.	Patient-Reported Assessments Versus Performance-Based Tests.....	413
	<i>Carolyn C. Gotay</i>	
44.	Alternative and Complementary Treatments	421
	<i>Barrie R. Cassileth and Christopher C. Chapman</i>	

V: Analyzing, Interpreting, and Presenting Data

45.	Quality of Life: Statistical Issues and Analysis	427
	<i>Diane L. Fairclough and Richard D. Gelber</i>	
46.	The Q-TWiST Method	437
	<i>Richard D. Gelber, Bernard F. Cole, Shari Gelber, and Aron Goldhirsch</i>	
47.	Interpreting General Health Measures	445
	<i>John E. Ware, Jr. and Susan D. Keller</i>	
48.	Clinical Significance of Quality of Life Data	461
	<i>Eva G. Lydick and Robert S. Epstein</i>	
49.	Evaluating and Comparing Life Events Data with Quality of Life Data	467
	<i>Bert Spilker</i>	
50.	Presentation of Quality of Life Data	477
	<i>John R. Schoenfelder and Bert Spilker</i>	

VI: Special Perspectives on Quality of Life Issues

51.	Quality of Life Assessments in Clinical Trials: An Ethical Perspective	489
	<i>Robert J. Levine</i>	
52.	Quality of Life Measures in Health Care and Medical Ethics	497
	<i>Dan Brock</i>	
53.	Cultural Considerations	511
	<i>Thomas M. Johnson</i>	
54.	The Role of Quality of Life Assessments in Medical Practice	517
	<i>Albert W. Wu and Kathleen A. Cagney</i>	
55.	Anthropological Perspectives: The Importance of Culture in the Assessment of Quality of Life	523
	<i>Peter J. Guarnaccia</i>	
56.	Quality of Life: The Nursing Perspective	529
	<i>Pamela S. Hinds and Claudette G. Varricchio</i>	
57.	An Industry Perspective	535
	<i>Bert Spilker and Stanley B. Garbus</i>	
58.	A Marketing Perspective: Theoretical Underpinnings	541
	<i>Louis A. Morris, Thomas K. Beckett, and Karen J. Lechter</i>	
59.	A Marketing Perspective: Practical Applications	549
	<i>Thomas K. Beckett</i>	
60.	An Epidemiologic Perspective	555
	<i>David S. Sugano and Newell E. McElwee</i>	
61.	Legal Considerations in the Development and Use of HRQL Measures for National and International Clinical Trial Research	563
	<i>Richard A. Berzon and John M. Conley</i>	
62.	Regulatory Perspectives on Quality of Life Issues	569
	<i>Mary S. McCabe, Dale Shoemaker, Robert J. Temple, Gregory Burke, and Michael A. Friedman</i>	

VII: Cross-Cultural and Cross-National Issues

- | | | |
|-----|---|-----|
| 63. | Language and Translation Issues
<i>Catherine Acquadro, Bernard Jambon, David Ellis, and
Patrick Marquis</i> | 575 |
| 64. | Quality of Life in Ethnic Groups.....
<i>Janis F. Hutchinson</i> | 587 |
| 65. | Assessing Health-Related Quality of Life in Disadvantaged and Very Ill
Populations.....
<i>Margot K. Ettl, Ron D. Hays, William E. Cunningham,
Martin F. Shapiro, and C. Keith Beck</i> | 595 |
| 66. | Conceptual Issues and Considerations in Cross-Cultural Validation of Generic
Health-Related Quality of Life Instruments
<i>Roger T. Anderson, Mary McFarlane, Michelle J. Naughton, and
Sally A. Shumaker</i> | 605 |
| 67. | International Use and Application of Generic Health-Related Quality of Life
Instruments.....
<i>Roger T. Anderson, Neil K. Aaronson, Alain P. Leplège, and
David Wilkin</i> | 613 |
| 68. | Dimension-Specific Instruments That May Be Used Across Cultures
<i>Michelle J. Naughton and Ingela K. Wiklund</i> | 633 |
| 69. | Creating and Evaluating Cross-Cultural Instruments
<i>Monika Bullinger, Michael J. Power, Neil K. Aaronson,
David F. Cella, and Roger T. Anderson</i> | 659 |
| 70. | A Mini-Handbook for Conducting Small-Scale Clinical Trials in Developing
Countries
<i>Harvey Schipper, Charles L. M. Olweny, and Jennifer J. Clinch</i> | 669 |
| 71. | Evaluating Instruments Used Cross-Nationally: Methods from the
IQOLA Project.....
<i>John E. Ware, Jr., Barbara L. Gandek, Susan D. Keller, and the
IQOLA Project Group</i> | 681 |
| 72. | Applying Quality of Life Principles in International Cancer Clinical Trials.....
<i>Jürg Bernhard, Christoph D. T. Hürny, Alan Coates, and
Richard D. Gelber</i> | 693 |
| 73. | Cross-Cultural Instrument Equating: Current Research and Future Directions
<i>David F. Cella, Stephen R. Lloyd, and Benjamin D. Wright</i> | 707 |

VIII: Health Policy Issues

- | | | |
|-----|--|-----|
| 74. | Applications of Health Status Assessment to Health Policy
<i>Donald L. Patrick and Pennifer Erickson</i> | 717 |
| 75. | Ethical and Medical Basis of Health Care Rationing
<i>Stefan Björk</i> | 729 |
| 76. | Using Decision-Analysis Approaches to Integrate QOL and Cost Data in Drug
Therapy Selection
<i>William F. McGhan</i> | 741 |

77.	Outcomes Research and Quality of Care.....	747
	<i>Donald M. Steinwachs, Albert W. Wu, and Kathleen A. Cagney</i>	
78.	Health Policy Implications of Using Quality of Life Measures in the Economic Evaluation of Health Care.....	753
	<i>Alan H. Williams</i>	
79.	Rules for Evaluating Medical Technologies.....	761
	<i>David M. Eddy</i>	
80.	International Price Comparisons	773
	<i>Patricia M. Danzon</i>	
81.	Roles of Government Agencies in Support and Application of Quality of Life Research.....	779
	<i>Susan G. Nayfield</i>	

IX: Special Populations to Assess Quality of Life

82.	Measuring Health-Related Quality of Life in Pediatric Populations: Conceptual Issues.....	785
	<i>Peter L. Rosenbaum and Saroj Saigal</i>	
83.	Measuring Health Outcomes in Pediatric Populations: Issues in Psychometrics and Application.....	793
	<i>Jeanne M. Landgraf and Linda N. Abetz</i>	
84.	Geriatrics: Perspective on Quality of Life and Care for Older People.....	803
	<i>T. Franklin Williams</i>	
85.	Frail Older Patients: Creating Standards of Care.....	809
	<i>Marsha D. Fretwell</i>	
86.	Measuring Health-Related Quality of Life in Older and Demented Populations.....	819
	<i>Anita L. Stewart, Cathy D. Sherbourne, and Meryl Brod</i>	
87.	Substance Abuse Disorders.....	831
	<i>David S. Metzger, Richard F. Davis, and Charles P. O'Brien</i>	
88.	Rehabilitation: Issues in Functional Assessment.....	839
	<i>Ralph R. Turner</i>	
89.	Defining Quality of Life in Chronic Pain	853
	<i>Victor C. Lee and John C. Rowlingson</i>	
90.	Quality of Life after Coronary Revascularization Procedures.....	865
	<i>Carol E. Cornell, James M. Raczynski, and Albert Oberman</i>	

X: Specific Problems and Diseases

91.	Cardiovascular Disorders.....	883
	<i>Nanette K. Wenger, Michelle J. Naughton, and Curt D. Furberg</i>	
92.	Hypertension	893
	<i>Ingela K. Wiklund</i>	
93.	Neurologic Illness.....	903
	<i>Robert S. Wilson, Christopher G. Goetz, and Glenn T. Stebbins</i>	

94.	Quality of Life Assessment for People with Epilepsy.....	909
	<i>Joyce A. Cramer</i>	
95.	Severe Mental Illness in the Community	919
	<i>Anthony F. Lehman and Barbara J. Burns</i>	
96.	Inflammatory Bowel Disease	925
	<i>Douglas A. Drossman</i>	
97.	Quality of Life Assessment in Gastrointestinal Surgery.....	937
	<i>Robin S. McLeod</i>	
98.	Measuring Disease-Specific Quality of Life in Men with Benign Prostatic Hyperplasia	945
	<i>Harry A. Guess</i>	
99.	Determining Quality of Life in the Renal Replacement Therapies.....	953
	<i>Atara Kaplan De-Nour and Andrew L. Brickman</i>	
100.	Quality of Life in Chronic Obstructive Pulmonary Disease.....	961
	<i>A. John McSweeney and Karen T. Labuhn</i>	
101.	Asthma and Allergy.....	977
	<i>Elizabeth F. Juniper and Gordon H. Guyatt</i>	
102.	Diabetes Mellitus	983
	<i>Richard S. Beaser, Sharon B. Garbus, and Alan M. Jacobson</i>	
103.	Cancer: Psychosocial Aspects	993
	<i>Ivan Barofsky</i>	
104.	Incorporating Quality of Life Assessment into Clinical Cancer Trials.....	1003
	<i>Michael D. E. Goodyear and Michael A. Fraumeni</i>	
105.	Quality of Life in Bone Marrow Transplant	1015
	<i>Betty R. Ferrell, Marcia M. Grant, and Marie B. Whedon</i>	
106.	Chronic Rheumatic Disorders	1029
	<i>Elizabeth W. Karlson, Jeffrey N. Katz, and Matthew H. Liang</i>	
107.	Quality of Life in Orthopaedics	1039
	<i>James G. Wright</i>	
108.	Measurement of Visual Functioning and Health-Related Quality of Life in Eye Disease and Cataract Surgery.....	1045
	<i>Carol M. Mangione, Paul P. Lee, and Ron D. Hays</i>	
109.	AIDS	1053
	<i>A. David Paltiel and Aaron A. Stinnett</i>	
110.	Nutrition and Quality of Life Measurement.....	1063
	<i>Carol E. Smith and Susan V. M. Kleinbeck</i>	

XI: Pharmacoeconomics

111.	Relationship of Pharmacoeconomics and Health-Related Quality of Life.....	1077
	<i>Dennis A. Revicki</i>	
112.	Designing and Conducting Cost-Benefit Analyses	1085
	<i>Magnus Johannesson and Milton C. Weinstein</i>	

113.	Designing and Conducting Cost-Minimization and Cost-Effectiveness Analyses.....	1093
	<i>Brian E. Rittenhouse</i>	
114.	Designing and Conducting Cost-Utility Analyses.....	1105
	<i>George W. Torrance</i>	
115.	Practical Aspects of Designing and Conducting Pharmacoeconomic Studies	1113
	<i>Andrew M. Baker</i>	
116.	Developing Guidelines for Pharmacoeconomic Trials.....	1123
	<i>Bert Spilker</i>	
117.	Users of Pharmacoeconomic Data	1131
	<i>Alan L. Hillman and Joseph A. Leveque</i>	
118.	Use of Pharmacoeconomic Data by Regulatory Authorities	1141
	<i>Paul P. Glasziou and Andrew S. Mitchell</i>	
119.	Use of Pharmacoeconomic Data by Pharmaceutical Companies	1149
	<i>Brian Lovatt</i>	
120.	Uses of Pharmacoeconomic Data by Policy-Makers and Pharmaceutical Benefit Managers.....	1153
	<i>Stephen W. Schondelmeyer</i>	
121.	Use and Opportunities for Administrative Databases in Pharmacoeconomic Research	1165
	<i>John E. Paul and Hugh H. Tilson</i>	
122.	Problems in Undertaking Pharmacoeconomic Assessments in Phase III Clinical Trials: The Case of Colony-Stimulating Factors.....	1173
	<i>Michael F. Drummond, Joseph Menzin, and Gerry Oster</i>	
123.	Comparing Pharmacoeconomic Data from Different Clinical Trials	1179
	<i>Flemming Ørnkov and Bengt G. Jönsson</i>	
124.	A Bayesian Approach to the Economic Evaluation of Health Care Technologies	1189
	<i>David A. Jones</i>	
125.	Using Decision Analysis to Conduct Pharmacoeconomic Studies.....	1197
	<i>Judith T. Barr and Gerald E. Schumacher</i>	
126.	Threats to the Validity of Pharmacoeconomic Analyses Based on Clinical Trial Data	1215
	<i>Brian E. Rittenhouse and Bernie J. O'Brien</i>	
127.	The Future of Pharmacoeconomics.....	1225
	<i>Michael F. Drummond</i>	
	Subject Index	1229
	Instruments Index	1255