

SCIENCE AND POLICY

Opinion



DENNIS A. REVICKI, Ph.D.

Patient-Based Outcomes Research in Europe: An Important Goal and Unique Challenge

Conducting patient-based outcomes studies in Europe

is associated with several inherent challenges. Multi-center, multi-country clinical trials are often conducted to evaluate the safety and efficacy of new medical treatments. Clinical centers from the United States, Canada, the United Kingdom, France, Germany, and other European countries are included in these clinical trials, and it is necessary to ensure that the patient-based outcome assessments have evidence of linguistic and cultural equivalence and acceptable psychometric qualities. Ideally, health-related quality of life instruments and other patient-based assessments are subjected to adequate procedures for translation and cultural validation, with pre-testing to obtain data on psychometric characteristics. We have been involved with several research partners in the development of instruments for multi-country applications.

Methods for collecting health-related quality of life and other patient-based outcomes may be more acceptable in some countries than in others. For example, telephone interviews and interactive voice response systems are very acceptable in the United States, but may not be acceptable in the United Kingdom or European countries. However, mail surveys may be very effective in the United Kingdom and European countries, while not very effective in the United States. The design and conduct of patient-based outcomes studies must consider cultural differences and sensitivities between

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MEDTAP International Appoints Peter B. Malamis Vice President of Strategic Initiatives

MEDTAP International has appointed Peter B. Malamis as Vice President, Strategic Initiatives.



PETER B. MALAMIS

In this newly created position, Mr. Malamis will work closely with MEDTAP's research scientists to apply their more than 15 years of leading outcomes and health economics research to challenges facing the healthcare marketplace.

Mr. Malamis' initial responsibilities are twofold: building relationships with pharmaceutical product marketing personnel in the major pharmaceutical companies to complement those already in place with clinical and outcomes specialists; and leading a new venture focused on integrating patient preferences into the prescribing process.

MEDTAP CEO Dr. Bryan R. Luce explained, "Mr. Malamis brings as many years of pharmaceutical and health care product marketing experience as we have on the outcomes research side. Our intent is to combine his expertise and experience with ours in order to develop applications and solutions that will enable pharmaceutical marketers to enhance communication with patients, physicians, managed care, and other insurers."

Mr. Malamis was founder and CEO of SYNTHESIS Pharmaceutical Programs, a medical marketing consulting company located in Alexandria, Virginia. He also serves as a board member for OUTCOMES International of Basle, Switzerland. From 1996-1999, he was at PAREXEL International, a major U.S.-based CRO, where he served as Corporate Vice President and Executive Vice President, North America Medical Marketing Services. From 1993-1996 he was an owner and Senior Vice President of State & Federal Associates, Inc. Mr. Malamis received his MBA from George Washington University and a BA in Political Science from the University of Richmond. ■

Executive and Management Workshops: Plan, Build, and Communicate The Value Message

OUTCOMES 2001...AND BEYOND

A unique, highly participative all-day workshop designed specifically for *clinical, regulatory and marketing* personnel to gain understanding and support of the health and economic outcomes research (HEOR) mission and program. Using the WORKMATS™ program, at the end of the day, participants ‘get it!’ They get why HEOR is important, what it is, what their respective role is, and how HEOR can help accomplish their own mission (to develop and communicate to the customer the value of the company’s products). Training is facilitated by both MEDTAP and client’s HEOR staff.

COMMUNICATING THE VALUE MESSAGE IN THE U.S.: A WORKSHOP FOR SENIOR MANAGEMENT

This one day off-site program is for *domestic senior director level management and direct reports from HEOR, Regulatory Affairs, Marketing, and selected Product Managers*. Integrating presentations with discussion, this program focuses on both the developing FDA regulation on quality-of-life claims and the four legal authorities for communicating the health economics message to the marketplace. Objectives are to: (1) develop a commonality of purpose across the three groups; (2) understand opportunities and challenges in communicating the value message to the market; and (3) enhance the process of developing and clearing these messages. MEDTAP senior scientists, Drs. Bryan Luce, Nancy Kline Leidy, and a nationally recognized former FDA official lead this workshop.

COMMUNICATING THE VALUE MESSAGE: AN EXECUTIVE SEMINAR FEATURING NICE AND MCO GUIDELINES IN THE U.S.

This 4-hour interactive seminar is for *senior company executives*. It addresses strategic implications of pharmaceutical economic guidelines emanating from the U.K.’s National Institute for Clinical Excellence (NICE); upcoming Blue Cross Blue Shield’s RxIntelligence evidence-based decision-making program and the early discussions of a ‘Euro-NICE’. MEDTAP senior scientists Bryan Luce and John Hutton who have had a leading role, respectively, in developing managed care and NICE guidelines lead this seminar.

For more information, contact Clark Paramore at 301.654.9729.

MEDTAP Welcomes New Scientific Personnel

In response to increased demand for its outcomes and health economic research services, MEDTAP has added several new scientific staff members with a variety of academic backgrounds.

DEBORAH L. JONAS, PH.D. — Dr. Jonas has joined MEDTAP as a research scientist in the Bethesda, Maryland office. Her responsibilities include the



DEBORAH L. JONAS

design and implementation of clinical studies involving patient-centered outcomes. “Dr. Jonas brings to MEDTAP a wonderful combination of education and experience,” said Bryan R. Luce, Ph.D. and CEO of MEDTAP. “Her background in cognitive psychology includes an in-depth understanding of standard neuro-psychological tests and their use in evaluating

cognition, which is critical in much of the patient-centered outcomes today, and her experience in clinical research management enables her to apply that understanding effectively when designing studies.”

Dr. Jonas holds a bachelor’s degree in psychology from the University of Maryland, a master’s degree in experimental psychology from Lehigh University, and a doctorate in cognitive psychology from Duke University. She has presented scientific papers at the annual conferences of the Psychonomic Society, as well as the Eastern Psychological Association. Dr. Jonas’ papers have appeared in *Psychology and Aging*, and *S.R. Waldstein and M.F. Elias’s (Eds) Neuropsychology of Cardiovascular Disease* (2000).

Prior to joining MEDTAP, Dr. Jonas designed and implemented studies assessing the potential differences between forward and backward serial recall, the effects of circadian rhythm on cognitive functioning, the role of spatial information in memory, and the effects of distraction on memory and attention in older and younger adults as well as children. In addition, Dr. Jonas was a clinical trials manager for ClinTrials Research in Cary,

North Carolina, where she was responsible for managing multiple research sites, recruiting and training investigators, and performing analyses to ensure data validity.

MANISHI PRASAD, M.P.H. — Manishi Prasad has joined MEDTAP as a project manager in the Bethesda, Maryland office, with responsibilities for managing and implementing research projects in both health outcomes and health



MANISHI PRASAD

economics, including data analysis, design and analysis of clinical trials, and report and manuscript writing.

Prior to joining MEDTAP, Ms. Prasad was a research associate with the Corporate Executive Board, Washington, DC, where she conducted strategic research for senior sales executives in Fortune 500 firms. At the

University of Michigan, Ms. Prasad performed research on a pancreatic cancer study, for which she assisted in laboratory research, conducted clinical literature searches, and arranged interviews with study participants.

Ms. Prasad holds a master of public health from the University of Michigan with a focus on health policy and epidemiological research. Her thesis was entitled “*Helicobacter pylori*: A Possible Risk for Pancreatic Cancer.” She earned a bachelor’s degree in economics from Wellesley College.

MARY KAY MARGOLIS, M.H.A./M.P.H. — Mary Kay Margolis has joined MEDTAP as a project manager in the Bethesda, Maryland office, where she is responsible for managing quality of life and other patient reported outcomes projects including instrument development and evaluation studies.

Prior to joining MEDTAP, Ms. Margolis was the manager of clinical research for the University of Pittsburgh School



MARY KAY MARGOLIS

of Medicine’s Department of Emergency Medicine. She was responsible for administering policies and procedures for conducting clinical research in the Department of Emergency Medicine, attracting public and private funding of research studies, preparing and administering budgets for research projects, and managing/monitoring all

clinical research conducted in the department. Prior to that, Ms. Margolis performed auditing and coordination of clinical research for the School of Medicine’s Division of Cardiology.

Ms. Margolis holds a master of health administration/ master of public health from the University of Pittsburgh’s Graduate School of Public Health and a bachelor’s degree in Exercise Science from the University of Pittsburgh.

TARA L. JONES, M.P.H. (Candidate) — Tara Jones has joined MEDTAP International as a project manager in the Bethesda, Maryland office. A specialist in epidemiology, Ms. Jones is responsible for managing and implementing research projects in both health outcomes and health economics, including data analysis, design and analysis of clinical trials, and report and manuscript writing.

Prior to joining MEDTAP, Ms. Jones was a research assistant in the Diabetes Prevention Program of the



TARA JONES

Biostatistics Center, George Washington University. She was responsible for management of clinical trial centers, including design and management of data collection, monitoring of site compliance with protocols and GCP guidelines, and tracking of study progress. As part of the African Development Aid Medical Project, Ms. Jones performed HIV/AIDS

curriculum development and conducted health-related workshops and surveys in Kenya, East Africa. In addition, Ms. Jones served as a clinical research specialist on studies of HIV, women’s needs, and geriatric health for the University of Illinois at Chicago. Ms. Jones holds a bachelors degree in chemistry from Spelman College and is a masters in public health candidate at the George Washington University.

GABRIELLE CIESLA, M.SC. — Gabrielle Ciesla has joined MEDTAP as an assistant project manager in the Bethesda, Maryland office. A specialist in medical informatics with experience in a variety of software



GABRIELLE CIESLA

languages (SAS, HTML, S-Plus), she is responsible for programming and analyzing large databases for economic studies, as well as interactive Web programming for data collection.

Prior to joining MEDTAP, Ms. Ciesla performed research and reporting on community health benchmarking for the Institute for Quality Health of Virginia.

In addition, she was responsible for designing an Access database of comprehensive mortality data for the state of Virginia, part of the VaLiance Community Health

Benchmarking Initiative. Ms. Ciesla holds a bachelor's degree in biology from West Virginia University and a master of science in health evaluation sciences from the University of Virginia.

TIMOTHY BAKER, B.S. — Timothy Baker has joined MEDTAP as a research assistant in the Bethesda, Maryland office. His responsibilities include support for outcomes and health economics research, including literature and Web searches, data collection and analysis, information organization, and generation of reports.



TIMOTHY BAKER

Mr. Baker's prior work experience included various literature reviews, data entry and analysis, and acting as a teaching intern on the intermediate level in the department of psychology,

University of Syracuse. Mr. Baker holds a bachelor's degree in psychology from Syracuse University.

LIA MARIE SNYDER, B.A. — Lia Snyder has joined MEDTAP as a research assistant in the Bethesda, Maryland office. Her responsibilities include conducting literature searches, Web searches, developing data tables and spreadsheets, and assisting in data collection and organization.



LIA MARIE SNYDER

Prior to joining MEDTAP, Ms. Snyder performed health administration functions for the University of Rochester Health Service and nursing assistant duties for the Genesee Hospital in Rochester, New York. Ms. Snyder holds a bachelor's degree in linguistics with a minor in biology from the University of Rochester, New York. ■

RECENT PUBLICATIONS

MEDTAP International welcomes comments and inquiries about our scientific work. Each issue, we feature one or more abstracts and a list of current publications. If you wish to obtain reprints of papers or a complete publication list, please contact Rebecca Sergi at 301.654. 9729.

Ejection Fraction and Health-Related Quality of Life — Demonstrating a Relationship

COYNE KS, LUNDERGAN CE, BOYLE D, GREENHOUSE SW, DRAOUI MS, WALKER P, ROSS AM. "Relationship of infarct artery patency and left ventricular ejection fraction to health-related quality of life after myocardial infarction. *Circulation* 2000;102:1245-1251.

Post-myocardial infarction global ejection fraction (EF) and infarct-related artery patency might be expected to be associated with HRQL outcomes, but this association has not been previously shown. The GUSTO-I Angiographic Study cohort 2-year follow-up afforded an examination of such potential relationships.

Conclusions: This is the first study to demonstrate a significant relationship between EF and long-term HRQL outcomes. This advantage in left ventricular function preservation should be added to the mortality advantage when considering the impact of treatment strategies for myocardial infarction.

BONOMI AE, SHIKIAR R, LEGRO MW. Quality-of-life assessment in acute, chronic, and cancer pain. A pharmacist's guide. *J Am Pharm Assoc* 2000; 40:402-416.

HAMILTON SH, EDGEL ET, REVICKI DA, BREIER A. "Functional outcomes in schizophrenia: a comparison of olanzapine and haloperidol in a European sample." *International Clinical Psychopharmacology* 2000; (15): 245-255.

KAPLAN MACHLIS B, SPIEGLER GE, ZODET MW, REVICKI DA. Effectiveness and costs of omeprazole vs ranitidine for treatment of symptomatic gastroesophageal reflux disease in primary care clinics in West Virginia. *Arch Fam Med* 2000; 9:624-630.

LEIDY NK, FARUP C, RENTZ AM, GANOCZY D, KOCH KL. "Patient-based assessment in dyspepsia development and validation of dyspepsia symptom severity index (DDSI)." *Digestive Diseases and Sciences* 2000; 45(6):1172-1179.

LEIDY, NK GUEST EDITORIAL: "Interpreting health-related quality-of-life outcomes." *Applied Clinical Trials*, 9 (9), 26.

Mavissakalian MR, Schmier JK, Flynn FA, Revicki DA. "Cost-effectiveness of acute imipramine therapy versus two imipramine maintenance treatment regimens for panic disorder." *PharmacoEconomics* 2000; 18:(4): 383-391.

OUSLANDER JG, SHIH YCT, MALONE-LEE J, AND LUBER KM. Overactive bladder: special considerations in the geriatric population. *American Journal of Managed Care* 2000; 6(11):S599-606.

PASCAL LECOMTE, MARC DE HERT, MARC VAN DIJK, MARK NUIJTEN, GUY NUYTS, ULF PERSSON. A 1-year cost-effectiveness model for the treatment of chronic schizophrenia with acute exacerbations in Belgium. *Value in Health*. 2000, Volume 3; Number 1: 1-11.

TAYLOR LA, SORENSEN SV, RAY NE, HALPERN MT, HARPER DM. Cost-effectiveness of the conventional papanicolaou test with a new adjunct to cytological screening for squamous cell carcinoma of the uterine cervix and its precursors. *Arch Fam Med* 2000; 9:713-721.

RECENT PRESENTATIONS & POSTERS

4TH ANNUAL SCIENTIFIC MEETING OF THE HEART FAILURE SOCIETY OF AMERICA, SEPTEMBER 2000

POSTER PRESENTATION:

- “Resource Use and Treatment Costs in a Heart Failure Patient Cohort” Gregory de Lissoy, Marc Zodet

7TH ANNUAL CONFERENCE OF THE INTERNATIONAL SOCIETY FOR QUALITY OF LIFE RESEARCH, VANCOUVER, BRITISH COLUMBIA, OCTOBER 2000

POSTER PRESENTATIONS:

- “Does Order of Administration Influence Subject Response on Generic and Condition-Specific Measures of Health-Related Quality of Life?” Nancy Kline Leidy PhD, Marcia Legro PhD, Jordana Schmier MA, Teresa Zyczynski PharmD, Colleen McHomey PhD, Karin Coyne PhD
- “Development and Psychometric Validation of the Patient Assessment of Upper Gastrointestinal Disorders – Symptom Severity Index (PAGI-SYM) in Dyspepsia” Jordana Schmier, Anne Rentz, Christina de la Loge, Dominique Dubois, Robert Jones, Dennis Revicki
- “Development and Psychometric Validation of the Patient Assessment of Upper Gastrointestinal Disorders — Symptom Severity Index (PAGI-SYM) in GERD” Anne Rentz, Jordana Schmier, Christina de la Loge, Dominique Dubois, Robert Jones, Marc Zodet, Dennis Revicki
- “Developing A Symptom and Health-Related Quality of Life Questionnaire for Uterine Fibroids” Karin Coyne PhD, Medha Sasane MS, Sheila Walsh MA, James Spies MD
- “Validating a Health-Related Quality of Life Questionnaire for Overactive Bladder From a Community Sample” Karin Coyne PhD, Walter Steward PhD, Jordana Schmier MA, Joshua Liberman PhD, Ron Corey PhD, Tim Hunt PhD, Paul Abrams MD, Dennis Revicki PhD
- “Effect of Mode of Administration on Symptom and Quality of Life Assessments in Gastrointestinal Disorders” Karin Coyne, Jordana Schmier, Dominique Dubois, Robert Jones, Anne Rentz, Dennis Revicki

INTERNATIONAL SOCIETY OF PHARMACOECONOMIC AND OUTCOMES RESEARCH, 3RD ANNUAL EUROPEAN CONFERENCE, ANTWERP BELGIUM, NOVEMBER 2000

CONTRIBUTED PODIUM PRESENTATION:

- “Patients’ reported health status and utilities for amyotrophic lateral sclerosis” Green C, Kiebert GM, Murphy C, Leigh PN, Mitchell JD, Burrell A.

CONTRIBUTED WORKSHOPS:

- “Appraisal of health technologies in England and Wales; Getting the submissions right” Hutton J, Taylor R, Chambers M, Gandhi G.

- “Economic evaluation of pharmaceuticals: The fourth hurdle and beyond” Hutton J, Nuijten M, Chambers M.

POSTER PRESENTATIONS:

- “A cost-cost study comparing etanercept with infliximab in moderate to severe rheumatoid arthritis” Nuijten MJC, Engelfriet PM, Duijn KJ, Wierz D, Koopmanschap M.
- “Budgetary impact analysis for use in reimbursement process of etanercept in moderate to severe rheumatoid arthritis” Huijten MJC, Engelfriet PM, Duijn KJ, Wierz D, Kiebert G.
- “Development and preliminary psychometric validation of the patient assessment of upper gastrointestinal disorders symptom severity index (PAGI-SYM) in GI patients” Rentz A, Schmier J, de la Loge C, Dubois D, Jones R, Peeters K, Zodet M, Revicki D.
- “Development and initial psychometric validation of the patient assessment of upper gastrointestinal disorders — quality of life instrument (PAGI-QOL) in GI patients” de la Loge C, Rentz A, Dubois D, Jones R, Peeters K, Marquis P.
- “Development and preliminary validation of the patient assessment of upper gastrointestinal disorders — symptom severity index (PAGI-SYM) in gastroparesis” Schmier J, Rentz A, de la Loge C, Dubois D, Jones R, Peeters K, Revicki D.
- “Cost-effectiveness analysis of entacapone in advanced Parkinson’s disease, a Markov process analysis” Nuijten MJC, van Iperen HP, Snyder E, Palmer C.
- “Projecting economic results of a European trial to the United States: issues and methods.” Sorensen S, de Lissoy G, Mathur S, Duttgupta S.

GERONTOLOGICAL SOCIETY OF AMERICA, 53RD ANNUAL SCIENTIFIC MEETING, WASHINGTON, DC, NOVEMBER 2000

SESSION: “MENTAL HEALTH AND DEMENTIA CARE”

Session Chair - Lori Frank, Ph.D.

PAPER:

- “Management strategies for neuropsychiatric behaviors in nursing homes: A task and cost analysis.” Kleinman L, Frank L, Schmier J, Rothman M.

POSTER PRESENTATIONS:

- “The patient experience of constipation: Testing the PAC with frail older adults in long-term care.” Flynn J, Frank L, Leidy NK, Rothman M.
- “Cost of nursing-based constipation care in nursing homes.” Frank L, Schmier J, Kleinman L, Siddique R, Bhattacharjya A, Rothman M. ■

Science and Policy Opinion...

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countries. This approach ensures that the highest quality data is collected within each clinical center and country.

Patient-based outcomes represents a broad category of endpoints, including health-related quality of life (physical, psychological, and social functioning and well-being), treatment adherence, treatment satisfaction, health state preference and utility, and caregiver burden. While European regulatory bodies do not require evidence of patient-based outcomes for registration of new drugs, reimbursement authorities in some countries have begun to recommend pharmacoeconomic evaluations, such as cost-effectiveness analysis, to inform the decision making process. An important component of cost-effectiveness analysis is the estimation of the relevant health-related outcomes. Several European countries (e.g., the United Kingdom, the Netherlands, France) are developing guidelines for the evaluation of cost-effectiveness and healthcare system budget impact for new pharmaceuticals.

Within the framework of these pharmacoeconomic guidelines, effectiveness is often measured within the context of clinical and patient-based outcomes. Clinicians and healthcare decision-makers need to compare the impact of competing medical treatments on patient health-related quality of life and other patient-based outcomes. This research answers the following question: Which therapy provides the best clinical and health outcomes from the patient's perspectives for the least cost? The value of patient-based outcomes depends on the ability of these assessments to assist in differentiating the effect of competing treatments on patient functioning and well-being in ways that are interpretable and meaningful to the clinical and healthcare system perspective.

Demonstrating clinical efficacy and safety endpoints is necessary but not sufficient in understanding the impact of treatment on patient health-related quality of life and other outcomes. You cannot assume that a specific clinical outcome, by definition, means that there is a consequent impact on patient health-related quality of life. We have a variety of reliable and valid instruments designed to directly measure the impact of both the treatment and the underlying disease on meaningful patient

outcomes. Generic and condition-specific instruments are available to measure patient functioning and well-being, symptoms, and health state preferences/utilities.

The U.S. Food and Drug Administration is developing guidelines for supporting evidence for new product labeling and promotional claims of benefit in patient-based outcomes, including health-related quality of life outcomes. At the same time, European regulatory authorities are considering standards of evidence for supporting statements regarding treatment benefits in patient-based outcomes.

Health-related quality of life and other patient-based outcomes reflect the impact of both the treatment and the disease on outcomes important to patients, their families and their physicians. These patient-based outcomes are increasingly incorporated into clinical trials. They are increasingly requested and used in decision-making by regulatory and health system authorities about the value and approval of new treatments, and by clinicians regarding the real benefit of different medical therapies from the patients' perspective.

The ultimate objective of healthcare interventions is the improvement or at least maintenance of health-related quality of life. This objective assumes survival and that survival without some evidence of life quality may not be enough. Survival alone is not a sufficient outcome of healthcare interventions. Evidence of the intrinsic value of healthcare therapies has become increasingly important as decision-makers across Europe grapple with the challenge of allocating healthcare resources in an equitable manner. The value of healthcare interventions depends on the perspective of the relevant stakeholder (i.e., patient, physician, healthcare system) and measured as clinical, economic, and patient-based outcomes. ■

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