

Please note: Workshops 4, 12, and 15 will be presented in Spanish. All other workshops will be presented in English. There will be no simultaneous translation of workshops.

2008 Workshops – October 22, 2008

Morning Workshops (1-8)

9:30 am to 12:30 pm

Workshop 1

Applying Item Response Theory (IRT) to Enhance Health Outcomes Assessment

Instructor: Bryce B. Reeve, National Cancer Institute, USA and Chih-Hung Chang, Northwestern University, USA

There is a great need in health outcomes research to develop instruments or assessment tools that can accurately measure a person's health status with minimal response burden. Further, these measurement tools must be free of bias due to possible cultural/racial differences which may affect responses to questionnaires administered in multi-national settings. This need for psychometrically sound and clinically meaningful measures calls for better analytical tools beyond the methods available from traditional measurement theory. Applications of item response theory (IRT) modeling have increased considerably because of its utility for instrument development and evaluation, assessment of measurement equivalence, instrument linking, item banking, and computerized adaptive testing. IRT models the relationship, in probabilistic terms, between a person's response to a survey question and their standing on a health construct (e.g., fatigue or depression) being measured. This information allows instrument developers to create reliable and efficient quality of life measures tailored for an individual or group or specific applications. This introductory workshop will first describe the basics of IRT models, then will focus in the second half of the session on the applications of these models to enhance health outcomes measurement. Illustrations from empirical data will be used throughout the presentation that focuses on measuring key health-related quality of life domains in different disease populations.

Level: Basic

Workshop 2

Advanced Psychometric Methods, Part 1: Use of Exploratory and Confirmatory Factor Analyses in PRO Instrument Development and Evaluation

Instructors: Donald E. Stull, and Margaret K. Vernon, United BioSource Corporation, USA

The development and psychometric evaluation of PRO instruments requires the application of a number of different techniques, including exploratory and confirmatory factor analysis (FA), item response theory analysis, and structural equation modeling (SEM). We will provide a brief overview of psychometric analyses and will then focus on the application of (1) exploratory and confirmatory factor analysis for understanding of new measures and (2) use of SEM for testing construct validity. Exploratory and confirmatory FA can be used to examine the relationships among items with a PRO measure or among different domains or multiple PRO measures. These techniques are useful for understanding the internal structure of PRO instruments and for understanding construct validity. This half-day workshop will describe the main methods of FA and illustrate these methods with examples from the instrument development literature. SEM is a powerful analytic technique that combines FA and path analysis in a simultaneous, confirmatory approach. Using SEM, the researcher can specify and evaluate hypothesized relationships between observed and latent (unobserved) constructs as well as relationships among the latent variables. SEM can also estimate the reliability and validity of measurement models while explicitly modeling measurement error. A researcher specifies a measurement model and a structural model which specifies relationships among the latent variables to examine construct and criterion-related validity. If the observed covariances are consistent with the model-implied covariances, the researcher has evidence supporting

the construct validity of the PRO measure. This workshop will demonstrate the main methods, testing assumptions and criteria, and provide examples to illustrate the methods of SEM.

Level: Advanced

Workshop 3

The Utility approach to Assessing Health-Related Quality of Life

Instructors: David Feeny, Kaiser Permanente Northwest, USA and Maria J. Santana, University of Alberta, Canada

The Workshop will be an introductory-level presentation on the utility approach to assessing health-related quality of life. Topics will include the conceptual foundations, practical methods for the direct elicitation of preference scores (visual analogue scale [Feeling Thermometer], time tradeoff, and standard gamble), multi-attribute approaches (EQ-5D, Health Utilities Index [HUI], Short-Form 6D [SF-6D], and Quality of Well-Being Scale [QWB]), a review of evidence on reliability, validity, responsiveness, and the interpretation of utility scores, and examples of applications. The Workshop will include hands on experience in the direct assessment of utility scores and in completing questionnaires from several systems and the analysis and interpretation of the results. Applications will be drawn from diverse applications including osteoarthritis of the knee, total hip arthroplasty, acute lymphoblastic leukemia, rehabilitation, rheumatoid arthritis, and population health. Guidance on criteria for selecting a utility measure for a study will also be provided. The session will include interactive demonstrations and discussion and didactic presentations. Outline I. Demonstration of Administration of Selected Utility Measures II. Introduction and Conceptual Foundations III Direct and Multi-Attribute Approaches for Obtaining Utility Scores IV Reliability, Validity, Responsiveness, and Interpretation of Utility Scores V Results from Demonstration VI Summary and Synthesis; Criteria for Choosing a Utility Measure

Level: Basic

Workshop 4

Methods for Cross-Cultural, Development, Translation/Adaptation, and Evaluation of Health Outcomes (*in Spanish*)

Instructors: Ramona Lucas, Institut de l'Envel·liment, UAB, Spain and Benjamin Arnold, CORE ENH Research Institute, Brazil

Cross-cultural translation of existing instruments has become an essential component of research methodology in preparation for multinational clinical trials. However, to improve cross-cultural equivalence, it is important to incorporate an understanding of cross-cultural issues prior to beginning translation work. This workshop will cover: 1) cross-cultural instrument development including a comparison of sequential, parallel, and simultaneous approaches with a special focus on WHOQOL methods; 2) types of cross-cultural equivalence and possible threats to validity if equivalence is not achieved; 3) instrument translation and adaptation methodologies; and 4) use of qualitative evaluation methods such as cognitive interviewing techniques to assess linguistic validity and cross-cultural equivalence of translated questionnaires. Workshop structure will include about 60% lecture, 25% Q&A and 15% interactive exercises on translation methodology and cognitive interviewing. The workshop will be taught in Spanish.

Level: Basic

Workshop 5

Advancements in the theory and practical applications of Response Shift (RS)

Instructors: Sara Ahmed, McGill University, Canada, Sandra Nolte, University of Melbourne, Australia, Carolyn Schwartz, New England Baptist Hospital, USA, and Lena Ring, Uppsala University, Sweden

This workshop will highlight theoretical, methodological and clinical developments in response shift (RS) research.

Special attention will be paid to the evolution of RS theory and current methods for detecting RS. In addition to design approaches (i.e., the then-test), we will also describe and demonstrate with empirical data new developments in RS methods that have evolved in the last decade. Statistical methods will include Structural Equation Modeling, Latent Trajectory Analysis, and Classification and Regression Tree Modeling. Individualized methods, such as the SEIQOL, the Patient-Generated Index, and semi-structured interviews, will be presented. In addition, we will emphasize studies that compare across various approaches, and contrast the pros and cons of each approach. The discussion will draw heavily on workshop participants' current experiences and future research needs. Small group exercises and a panel discussion will help participants engage in the thinking process of patients when responding to health status questionnaires.

Level: Basic

Workshop 6

Introduction to scaling models for measuring HRQoL

Instructor: Paul Krabbe, Radboud University Nijmegen Medical Centre, The Netherlands

In current health outcomes research only two scaling models are used to measure HRQoL. The leading model is based on classical test theory; the other more recent model is the Rasch model. However, classical test theory is associated with many drawbacks, whereas the Rasch model is just one of the available models developed in item response theory. So far, many alternative, attractive scaling models have not been applied in the field or only scarcely. The aim of this workshop is to present and explain the framework of scaling models and to show their potential. In the social sciences, scaling is the process of measuring entities with respect to quantitative attributes or traits. Two response mechanisms of scaling can be distinguished: cumulative and unfolding mechanisms. For each of these two scaling mechanisms there are two basic data collection designs: direct response and comparison response. The most well-known members of cumulative-direct scaling models are the Likert scale, the visual analogue scale, and the Rasch model. In HRQoL research, the latter model is used mostly for the measurement of abilities. Cumulative-comparative scaling models include Thurstone scaling, members of the class of conjoint analysis, multidimensional scaling, and the modern work of discrete choice modeling. The unfolding models are most suited for the measurement of attitudes and preferences. Depending on the research question and the object of study these unfolding models, absent in present HRQoL research, may offer solutions to many HRQoL research issues. Outline of the workshop: I) Introduction of the conceptual ideas (no mathematics) behind scaling models and their relationships and possible contributions to the measurement of HRQoL. II) Demonstration of the models. III) Hands on experience with a basic scaling model in a class exercise. IV) Presenting results from own studies and other researchers. Handouts will include all slides and key publications. Workshop structure: 60% lecture, 20% interactive exercise, 20% discussion.

Level: Basic

Workshop 7

Introduction to the Analysis of Longitudinal Studies of Health-Related Quality of Life

Instructor: Diane L. Fairclough, University of Denver, USA

This workshop will provide an introduction to the analysis of longitudinal studies in which missing data can be considered ignorable or missing at random (MAR). We will describe strategies for distinguishing between repeated measures models and growth curve models. Examples will be given for testing hypotheses for both simple and complex models including strategies for inclusion of time-varying covariates and interactions. We will discuss the use of polynomial and piecewise linear models. Finally we will discuss the concepts of moderation and mediation, and how to construct the appropriate models and tests. The focus of the workshop will be on interpretation and I will rely heavily on graphical presentations to convey concepts. Handouts will include all slides. The workshop will include discussion (20% of time). After completing this workshop, participants will be able to: 1. Understand the differences between a repeated measures and growth curve model for the analysis of

longitudinal data 2. Interpret the results of hypothesis tests for both simple and complex models 3. Distinguish the concepts of moderation and mediation Participants should have some experience with simple regression analysis.
Level: Basic

Workshop 8

Coping with the validation requirements of the FDA PRO Guidance: the Intersection of Science and Practice

Instructors: William R. Lenderking and Elizabeth Merikle, United BioSource Corporation, USA and Michelle Stewart, Pfizer, Inc., USA

This workshop will take off from the draft PRO guidance and consider the practical implications and scientific evidence supporting certain aspects of the guidance. In particular, we will focus on validation of patient reported outcome measures in light of the guidance on conceptual frameworks, conceptual models, and endpoint models. We will also consider the scientific evidence for the recommendations around the recall period. Practical, real-world examples/ research dilemmas will be presented for discussion, and we will consider alternative solutions, as well as discussing the solution ultimately chosen. Questions to be considered include the following: What does the literature say about validation requirements for a scale that has been in use for decades but has obvious limitations? What constitutes sufficient validation? If a scale has been validated in a given study population, what are the validation requirements for applying it to a new population, according to both the FDA and the literature? What if your research team wants to administer a questionnaire on a weekly basis, and it has only been previously developed for monthly administration? Are there different aspects of health and functioning which might be optimally measured over different recall periods? Can measures with different recall periods be compared within the same study? Does it make sense to assess functioning on a daily basis, or should daily measurement be more appropriately reserved for symptoms?

Level: Basic

Afternoon Workshops (9-16)

1:30 pm to 4:30 pm

Workshop 9

PROMIS Assessment Center Online Software for Clinical Research

Instructors: Nan E. Rothrock and Susan E. Yount, Evanston Northwestern Healthcare, USA

Assessment Center (www.assessmentcenter.net/ac1) is an online, dynamic application that allows researchers to centralize all research activities. Assessment Center houses a library of PROMIS health-related quality of life instruments and items. It includes features that allow end-users to conduct tasks such as instrument development, study administration, data management, and storage of statistical analyses. Assessment Center houses a library of instruments and items with an emphasis on health-related quality of life. Users are able to preview instruments as they would appear in data collection or preview CAT instruments with calculation details. Users may also download PDF versions of PROMIS instruments. Users can create their own instruments through selecting or modifying existing items and/or creating new items. Users may define data collection elements and create study-specific websites to administer instruments and consent forms. Data collection can be managed through accrual reports and participant registration screens. Assessment Center enables exporting study data to external applications for analysis. This workshop will train participants to use all current Assessment Center features through lecture, hands-on exercises, and question/answer time.

Level: Basic

Workshop 10

Advanced Psychometric Methods, Part 2: Executing and Interpreting Exploratory and Confirmatory Factor Analyses in PRO Instrument Development and Evaluation

Instructors: Donald E. Stull and Margaret K. Vernon, United BioSource Corporation, USA

This half-day workshop will present results from examples of exploratory and confirmatory factor analyses; executing live, interactive analyses; and interpreting results of output, particularly for confirmatory factor analyses and structural equation models. We will work through examples of analyses by presenting hypothesized models, discussing key analytic criteria (e.g., sample size, factor loading size, extraction, rotation, key parameter estimates, cross-loadings and correlated errors, model specification and identification, fit indices, indications of model misfit), and how to interpret output. Annotated examples will be presented from output from selected software (e.g., SAS, Stata, EQS, and Mplus), but the issues are relevant regardless of the users' software.

Level: Advanced

Workshop 11

Measuring Patient Satisfaction with Health Care

Instructor: Graeme Hawthorne, The University of Melbourne, Australia

Assessing patient satisfaction with health care is important for three reasons: it provides patients with a voice regarding their relationship with clinicians, it enables patient views to be taken into account during health care decision-making, and it assists with the monitoring of health care quality and the legitimization of health care policy. Despite the importance of eliciting this patient perspective, the assessment of patient satisfaction is often either left to the last minute (as a kind of afterthought) or is misdirected (through excessive questionnaires which may be intimidating for both patients and health care workers). This workshop is aimed at participants who wish to become more informed about the definition of patient satisfaction and its measurement in a way that is both practical and informative. The workshop will introduce the major patient satisfaction theories and will provide a review of the leading patient satisfaction questionnaires, including single assessment items and multi-scaled instruments. Selected case studies will be presented, illustrating the different kinds of approaches used in the patient satisfaction field, and participants will have the opportunity to directly compare different patient satisfaction measures. Participants will be introduced to the Short Assessment of Patient Satisfaction (SAPS) instrument, a new user-friendly instrument of patient satisfaction developed to capture satisfaction with health care. By the end of the workshop participants will have a firm understanding of patient satisfaction theory and practice (including the underlying principles, scoring algorithms, strengths and limitations of the leading instruments), an introduction to some of the key literature, a working knowledge of the different uses to which patient satisfaction can be put, and sufficient understanding to be able to make informed judgements about the level of patient satisfaction assessment which should be used under differing circumstances (from individual consultation to system-wide quality assurance).

Level: Basic

Workshop 12

Development and uses of EMPRO: a tool for the standardized evaluation of Patient Reported Outcome measures (in Spanish)

Instructors: Montserrat Ferrer, Jordi Alonso and Michael Herdman, IMIM-Hospital del Mar, CIBERESP, Spain

CONTENT: The workshop will focus on providing a description of the development and content of the EMPRO system, an online tool developed to standardize the appraisal of PRO instruments. Instruments assessed using EMPRO are judged on 8 key attributes (concept and measurement model, cultural adaptation, reliability, validity, sensitivity to change, interpretation, burden, alternative modes of administration) by a minimum of two expert appraisers using bibliographic information provided for the appraisal. After completing the initial 39 item

questionnaire individually, appraisers then participate in two rounds of consensus-building before final ratings for the instrument in question are calculated. **OUTLINE OF THE WORKSHOP:** After a brief introduction to the process of development of EMPRO, the workshop will focus on the 8 key attributes assessed by EMPRO as outlined above. For each attribute, a definition will be provided and the criteria used to assess it will be described. Items used to assess the attribute will be presented individually and specific examples from the literature will be used to illustrate the range of possible responses. The workshop will finish with an explanation of the scoring and interpretation of EMPRO and a practical example of how the tool is being used in an international exercise to evaluate PRO questionnaires for use in heart failure. **ORGANIZATION / STYLE OF PRESENTATION:** Approximately 60% of the workshop will be in lecture format, 20% will consist of a class exercise, and 20% will be in question and answer format. This workshop will be taught in Spanish.

Level: Basic

Workshop 13

A framework for specifying patient reported outcomes for complex health education interventions: making sure the right outcomes are assessed at the right time

Instructor: Richard H. Osborne and Sandra Nolte, Royal Melbourne Hospital, University of Melbourne, Australia

The choice of the right range of measures to capture the intended and unintended effects of interventions can be difficult for clinicians, trialists and researchers. If the right measures are not specified then the data collected will not provide a true reflection of the impact of an intervention (i.e., patient outcomes in a clinical trial) or the impact of a disease across the community. Clearly, a poor understanding and poor specification of the objective of an intervention or survey will lead to inadequate outcome measurement. Without a clear idea of what you are measuring, how can you measure it accurately? With the plethora of measures available and the wide range of possible intended impacts of interventions, including immediate and long term effects, a framework to support comprehensive but targeted assessment is warranted. This workshop will draw from several fields; quality of life, work health, chronic disease health education, as well as from design of clinical trials and population surveys. Consideration will be paid to a range of potential immediate impacts (e.g., education/knowledge), intermediate impacts (e.g., empowerment, life quality) and longer term impacts (e.g., use of health services, reduction of symptoms). This is a high energy, fast moving and participatory workshop. Attendees are encouraged to bring their current program to the workshop for *_work-shopping* so that the intended impact of their program can be explored and clarified and then a range of pertinent patient reported outcomes can be specified using the framework.

Level: Advanced

Workshop 14

Analysis of longitudinal studies of HRQOL in SAS and R

Instructor: Diane L. Fairclough, University of Denver, USA

This course will provide a tutorial of the analysis of longitudinal studies with missing data. We address two scenarios for data with ignorable dropout and two scenarios with non-ignorable dropout. For each scenario, we will first discuss the assumptions underlying each model, then will go through the steps of fitting the model in SAS and R. The workshop will include time for practice with datasets that will be provided. Participant are encouraged (but not required) to bring laptop computers with either SAS or R installed. Handouts will include all slides and example code in both SAS and R. After completing this workshop, participants will be able to: 1. Choose between a repeated measures and growth curve model for the analysis of longitudinal data 2. Describe the underlying assumptions of these models and alternative models for non-ignorable dropout 3. Build models and construct hypothesis tests in either SAS or R 4. Perform sensitivity analysis in settings with potential non-ignorable dropout Participants should have experience with linear models and be comfortable programming in either SAS or R.

Level: Advanced

Workshop 15

Practical difficulties and ethical considerations in quality of life research in patients with cancer (*in Spanish*)

Instructors: Veronica C. Aliaga, University of Chile, Chile and Paulina A. Araya, Universidad San Sebastian, Chile

Part 1. Analysis of practical difficulties in quality of life research in patients with cancer Theoretical exposition (30 minutes) Lecturer: Paulina Araya Part 2. Analysis of ethical considerations in quality of life research in patients with cancer Theoretical exposition (30 minutes) Lecturer: Verónica Aliaga Part 3. Discussion about the contents Applied exercise (30 minutes) Lecturer: Verónica Aliaga / Paulina Araya Part 4. Creation of an informed consent document for this specific population Applied exercise (30 minutes) Lecturer: Verónica Aliaga / Paulina Araya Part 5: Discussion about the documents Applied exercise (30 minutes) Lecturer: Verónica Aliaga / Paulina Araya
Level: Basic

Workshop 16

Evaluating Changes in Health-Related Quality of Life Measures and Other Patient-Reported Outcomes

Instructor: Kathleen W. Wyrwich, United BioSource Corporation, USA

Several strategies for identifying meaningful shifts in health-related quality of life (HRQoL) measures and other patient-reported outcomes (PROs) have emerged as key methods for interpreting, understanding and evaluating change over time. This workshop will review, critique and compare the methods that have been applied to establish meaningful change standards, such as the minimal important difference (MID), which include anchor- and distribution-based techniques. Practical approaches to improving and advancing HRQoL and PRO change evaluations that enhance the interpretation of change, as well as a review of controversies that have developed, will be provided. In addition, this workshop will explore current regulatory guidelines for demonstrating important change in HRQoL measures and other PROs.

Level: Advanced