RESULTS: Patient samples had a mean age of 60.20±14.84 years. Mean duration of dialysis was 7.4±5.42 years for hemodialysis patients, and 1.8±2.12 years for peritoneal-dialysis patients. The mean SF-6D score (0.783±0.164) was significantly higher for EQ-5D (UK) and Thai (0.752±0.164, and 0.691±0.196) scores. Most of the kidney specific dimensions were better correlated with SF-6D than EQ-5D (UK and Thai preference weight) and VAS scores. Ceiling effects were observed in the EQ-5D concerning both UK and Thai preference weight, due to the fact that the EQ-5D differentiates less in the better health states, whereas the floor effects were not clearly observed in any instrument tools.

CONCLUSIONS: SF-6D presented better correlation with kidney specific scales, while the responsiveness of EQ-5D utility scores was poor. One explanation might be a “ceiling effect” of the EQ-5D. These findings implied that SF-6D utility scores could reflect HRQoL status of dialysis patients better than EQ-5D and VAS.

PUE36

USING BOOTSTRAP CONFIDENCE INTERVALS TO COMPARE RELATIVE VALIDITY COEFFICIENTS: AN EXAMPLE WITH PRO MEASURES OF CHRONIC KIDNEY DISEASE (CKD) IMPACT

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OBJECTIVES: To evaluate bootstrap techniques in comparing the validity of PRO measures in discriminating among CKD patients and responding to longitudinal changes. METHODS: The Kidney Disease Impact Scale (KDIS), CKD-specific legacy (KDQOL-Burden, Symptom, and Effect) and generic health (SF-12) scales were administered to 453 patients and re-administered to 110 patients after three months. ANOVA-based relative validity (RV) coefficients were used to compare how well each scale discriminated between three clinically-defined groups ordered in terms of severity (Dialysis > Stage 3 > Transplant), and how responsive each scale was to changes over time for self-evaluated Better, Same and Worse groups. Bootstrap was used to construct confidence intervals (CIs) to determine whether the differences in RVs were significant in comparisons between each scale and the best legacy measure - KDQOL Burden. Sample size, number of bootstrap iterations, and type of CIs were varied to evaluate their impacts on CI using real and artificial data. RESULTS: The sample size played a substantial role. 300 people for 3 groups were suggested as the minimum number to make meaningful comparisons between RVs using CI. Number of bootstrap replications (100 to 10,000) did not show an obvious effect on bootstrap standard error, although 300 showed improvement over 100 on CI. The bias-corrected and accelerated (BCa) type of CI was preferred for correcting both bias and skewness in bootstrap distribution and for producing narrower CIs. Using 95% CI and 300 sample size, differences in RVs were non-significant in comparisons with KDQOL Burden (RV = 1) for the following scales: SF-12 PCS (RV = 0.6), FF (RV = 0.7), RF (RV = 0.7), KDQOL-Effect (RV = 0.9), and KDIS (RV = 1.13). CONCLUSIONS: Bootstrapping appears to be valuable in testing the significance of differences in PRO measures in chronic kidney disease patients.

RESEARCH POSTER PRESENTATIONS – SESSION IV

RESEARCH ON METHODS STUDIES

RESEARCH ON METHODS – Clinical Outcomes Methods

PBM1

COMPLIANCE ON THE CONSOLIDATED STANDARDS OF REPORTING TRIALS (CONSORT) GUIDELINES IN RANDOMIZED CONTROLLED TRIALS

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OBJECTIVES: The Consolidated Standards of Reporting Trials (CONSORT) statement was published in 2001 and updated in 2010, strongly recommended the use of CONSORT diagram to report the flow of participants through each stages of the trial. This study was conducted to describe the level of compliance of the published clinical trial in following the CONSORT recommendations and to estimate prevalence of the compliance. METHODS: A systematic literature search of all randomized controlled trials of anti-infectious agents published in the top 10 general medicine journals and top 5 infectious disease journals published in 2010. The journals included: The New England Journal of Medicine, Journal of the American Medical Association, British Medical Journal (Clinical Research Ed), Archives of Internal Medicine, PLoS Medicine, Annals of Internal Medicine, Clinical Infectious Diseases, The Journal of Infectious Diseases, the Lancet Infectious Diseases, AIDS, Emerging Infectious Diseases Journal, Annual Review of Medicine, Canadian Medical Association Journal, and Annals of Medicine Journal. Each article was reviewed by two independent investigators based on the reporting criteria recommended by the CONSORT statement. Exclusion criteria included non-randomized control studies, and studies not including intervention or control group. RESULTS: The study iden- tified 2103 relevant articles, and 246 randomized controlled trials met the inclusion criteria. Of 73 studies, 55 (75.34%) articles included the CONSORT diagram. A comprehensive depiction of the CONSORT guidelines will be made and detail descriptions on the compliances will be presented by journal types during the presentation. CONCLUSIONS: Randomized controlled trials published in the top 10 general medicine journals and the top 5 infectious diseases journals in 2010 contain significant deficiencies in reporting the CONSORT flow chart. The clarity and the completeness of a study could be improved if the CONSORT statement is followed as prescribed.

PBM3

NETWORK META-ANALYSIS OF INDIVIDUAL AND AGGREGATE LEVEL DATA

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OBJECTIVES: Network meta-analysis is often performed with aggregate level data (AD). A challenge with meta-regression models using AD is that the association between a patient level covariate and relative treatment effects of the compared interventions at the study level may not reflect the individual level effect-modification by the covariate. In this paper, we illustrate network meta-analysis models for combining individual patient data (IPD) and AD are presented to reduce bias and uncertainty of treatment effects in the presence of heterogeneity due to patient characteristics. METHODS: The first method uses the same model form for IPD and AD. With the second approach, the models are based on a non-linear interaction term and therefore seems less affected by bias. Additional studies, however, are needed to assess the value of both models. CONCLUSIONS: Overall, for network meta-analysis it is recommended to use IPD when available, rather than treating all studies as AD.

PBM4

THE ENSEMBLE MINIMUM DATABASE: A NEW INSTRUMENT TO EXPLORE HETEROGENEITY OF TREATMENT EFFECT

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OBJECTIVES: To develop an instrument that identifies patient groups likely to have differing responses to treatment, we tested candidate measures thought to discriminate differences among patients in 4 disease cohorts: type 2 diabetes (T2D), knee osteoarthritis (OA), ischemic heart disease (IHD) and heart failure (HF). METHODS: Eligible patients identified from claims data were sent a survey including 17 scales hypothesized to comprise 4 domains (health profile, personality, behavior, lifestyle). Proxies for treatment response were patient-reported global impression of disease severity (PGIS), global impression of improvement (PGII), and administrative claims health care utilization (HCI). Variability (SD) and internal consistency (Cronbach’s alpha) of the scales were examined, as discriminant validity against strata of PGIS, PGII and HCI. Conceptual overlap, correlations among scales, and factor loading within and across domains were examined. Scales with desirable properties were included in the final instrument. Discriminant validity of proposed domains was analyzed by ANOVA adjusted for age and gender. RESULTS: The study involved 482 patients with type 2 diabetes, 436 knee OA patients, 632 IHD patients, and 588 HF patients completed the survey. The initial instrument was refined to 7 scales across 3 domains. The health profile domain significantly discriminated 100% of the strata across disease cohorts (each P < 0.001). Correlation and behavior domains also discriminated strata well (75% and 50%, respectively). Apart from the health profile domain, discriminant validity against strata of PGIS, PGII and HCI was observed. CONCLUSIONS: The final ENSEMBLE MDI instrument discriminated among patients with varied diseases, the health profile provided much of the ability to discriminate. Further research is needed to assess the instrument’s potential to predict health state changes due to trial interventions.

PBM7

ENHANCING THE HEALTH ECONOMIC VALUE OF RETROSPECTIVE AND PROSPECTIVE REAL-WORLD STUDIES WITH PHARMACOGENOMIC TESTING: OPPORTUNITIES AND CHALLENGES ASSOCIATED WITH AN INTEGRATED PERSONALIZED MEDICINE APPROACH

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OBJECTIVES: A better understanding of a patient’s genetic make-up through pharmacogenomic testing can help achieve improved and more predictable patient outcomes, often at equal or lower total treatment cost. Stakeholders including physicians, payers and patients alike can benefit from real-world data that identify, predict health state changes due to trial interventions. RESULTS: A better understanding of a patient’s genetic make-up through pharmacogenomic testing can help achieve improved and more predictable patient outcomes, often at equal or lower total treatment cost. Stakeholders including physicians, payers and patients alike can benefit from real-world data that identify, predict health state changes due to trial interventions.