APPLICATION OF ZERO INFLECTED POISSON MODEL FOR ESTIMATING FUNCTIONS OF COST AND EFFECTIVENESS IN COUNT DATA

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OBJECTIVES: In continuous outcome variables to be measures of effectiveness in cost effectiveness/benefit analysis, it is very straightforward to estimate functions of cost and effectiveness such as incremental cost effectiveness ratio (ICER), and incremental net benefit (INB). As well as life expectancy or survival time, primary outcome variables in many clinical trials are count data. Nevertheless, estimation or inference procedure of those data is not well explained. Especially, measures of post treatment in count data includes excessive zero due to treatment effect. In this study, we provide and compare estimation strategies for functions of cost and effectiveness which consist of counts using Poisson, negative binomial, zero inflated Poisson model (including regression model) and explore the properties of each model.

METHODS: Point and interval estimation of ICER and INB for each model will be derived, and through the simulation study, we compare power to assess the performance in various situations. Illustrative example was presented with “Randomised, flexible dose and open study to compare the efficacy and safety of Adalat Oros with ginkgo biloba extract to treat the patients with the Raynaud’s disease”.

RESULTS: On the ground of power comparison and confidence intervals of ICER and INB, ordinal modeling of count data leaded to biased estimation of ICER and INB. Compared to zero inflated Poisson model of ICER and INB, simple estimation which count data were considered continuous variable underestimated more than 1.5 times. CONCLUSION: The study demonstrated the estimation procedure of cost and effectiveness functions and appropriate modeling of count data prevent biased estimate of ICER or INB.

PRICE VS ACCESS: A PROBABILISTIC MODEL FOR GUIDING PRICING DECISIONS

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OBJECTIVES: To produce a simple probabilistic model for guiding pharmaceutical pricing decisions, across multiple and various health care markets, in the presence of uncertainty regarding market-specific characteristics, access and share.

METHODS: Pharmaceutical pricing appears to be largely determined by the price levels within a drug class or indication with less emphasis placed on cost effectiveness and budgetary impact. Health payers by contrast are increasingly using cost-effectiveness and budgetary impact measures to determine whether a product should be reimbursed at the requested price. We set out a simple system for probabilistically modelling manufacturer revenues from a product as a function of product price. The system relies on explicit modelling of the reimbursement probability as a function of price, and of market share conditional on reimbursement as a function of price. Uncertainty can be incorporated at the patient and market level. A revenue probability distribution function is derived as a function of price.

RESULTS: For single market models, the system produces the familiar “all or nothing” results of lesser interest: lower prices equate to greater probabilities of reimbursement and market share. The system is most valuable in multi-market situations where relationships are more complex or in markets where, due to the presence of competitor products or reference pricing, the relationship with price of probability of acceptance or level of market share is complex. Results in these settings are less generalizable. We demonstrate this system in a number of illustrative single and multi-market settings. CONCLUSION: The revenue probability distribution function provides a useful tool to guide pharmaceutical pricing decisions, according to a company’s risk profile. The model developed facilitates decisions as to when and how to trade-off reimbursement probability for revenue per patient, with the aim of maximal revenues at a global level.

PREDICTORS OF SELF-RATED HEALTH STATUS AMONG GENERAL POPULATION IN THE UNITED STATES USING THE MEDICAL EXPENDITURE PANEL SURVEY (MEPS)

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OBJECTIVES: To investigate the predictors of self-rated health status for the U.S. civilian noninstitutionalized population.

METHODS: Cross-sectional analyses using multivariate logistic regression were performed with 11,405 individuals from the 2003 MEPS. The self-rated health status was dichotomized into two categories (fair/poor health and good/very good/excellent health). Standard demographic variables were employed as predictors in the regression, and the impact of a given predictor on the self-rated health status was obtained as odds ratios. To check the presence of multicollinearity, the conditional index and variance composition were examined.

RESULTS: Smaller family size, very low incomes (less than $15,000/year), non-white ethnicity (Hispanic, African-American, and Asian), older ages, female, lower education than college, having public health insurance, and having worse mental health status were significantly and consistently associated with fair/poor perceived health (p < 0.05).

An additional family member and male gender were related with decreased risk of fair/poor perceived health by 0.88 and 0.87, respectively. Subjects with very low incomes, Hispanic ethnicity, and aged between 56 to 65 were associated with 1.19, 1.34, and 3.65 times more likely to rate their health as fair/poor in comparison with subjects with high incomes (more than $100,000/year), White ethnicity, and aged between 16 to 25, respectively. Similarly, persons with lower education (high school degree) and public insurance would be 1.77 and 1.75 times as
likely as persons with Bachelor’s degree and without insurance to rate their health as fair/poor, respectively. An additional score of mental health status (1 [excellent] − 5 [poor]) was associated with 2.67 times more likely to have fair/poor perceived health. The model did not suffer from significant multicollinearity since the maximum conditional index was less than 10. CONCLUSION: Health perceptions are strongly associated with standard demographic variables like income, ethnicity and age. Future work should address the causal factors behind these associations.

A RETROSPECTIVE ANALYSIS OF PATIENT-REPORTED OUTCOMES AND OTHER EFFICACY ENDPOINTS IN TOP BRAND NAME PRODUCT LABELS IN UNITED STATES

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OBJECTIVES: Patient-reported-outcomes (PROs) are endpoints derived from the patient, collected by various means: verbally in a clinic, in a diary, event logs, symptom reports, or formal instruments, to measure health status, compliance, and satisfaction with treatment choices. This study determines the level of PROs reported for the brand name drugs in the Top 200 Products in the US Total Market by Dispensed Total Scripts (http://www.pharmacytimes.com), in comparison to other types of effectiveness endpoints, such as clinician-reported-outcomes (CROs) and laboratory values. METHODS: Brand name drugs with web based package inserts from a list of the top 200 drugs for 2005 were selected for inclusion in this study. Data collected included: brand and generic names, manufacturer, therapeutic class, and types and numbers of PROs, CROs, and laboratory endpoints present in the package insert. RESULTS: Eighty-six brand name drugs were included in the final analysis, with the highest percentages for antihypertensives and dyslipidemic agents. Patient-reported-outcomes (PROs) were reported in 10 (11.63%) of the package inserts reviewed. Clinician-reported-outcomes (CROs) were reported in 73 (84.88%) of the package inserts reviewed and laboratory outcomes were reported in 23 (26.74%) of the package inserts. Twenty-three separate formal PRO scales were utilized, with the most common ones present (26.74%) of the package inserts. Twenty-three separate formal PRO scales were utilized, with the most common ones present (26.74%) of the package inserts. Twenty-three separate formal PRO scales were utilized, with the most common ones present (26.74%) of the package inserts. Twenty-three separate formal PRO scales were utilized, with the most common ones present (26.74%) of the package inserts. 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A COMPARISON OF CLINICAL TRIAL PARTICIPANTS TO THE GENERAL PATIENT POPULATION IN FRANCE, GERMANY AND UK

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OBJECTIVES: To identify and describe differences in demographics, quality of life, resource utilization, and health care attitudes between clinical trials participants and the general population. METHODS: Data for this analysis were obtained from the 2005 National Health and Wellness Survey (NHWS), an annual nationally representative Internet-based study of the health status, health care attitudes, and behaviors of adults (age 18+). The current analysis was limited to respondents from France, Germany and the UK reporting a diagnosis of hypertension, high cholesterol, or diabetes (n = 8384). Respondents reported ever participating in a clinical drug trial, demographic, attitudes, resource use, and quality of life (SF-8 summary scores). RESULTS: Across all countries, 6% (n = 472) reported ever participating in a clinical drug trial (France, 4%; Germany, 6%; UK, 7%). In all countries, clinical trial participants were older (France, 59.19 vs. 52.26, p < 0.01; Germany, 56.30 vs. 52.64, p < 0.01; UK, 59.93 vs. 56.01, p < 0.01), and had more frequent physician visits (France, 9.17 vs. 1.01, p = 0.01; Germany, 10.27 vs. 7.47, p < 0.001; UK, 6.51 vs. 5.60, p = 0.03). Clinical trials participants in France and Germany reported lower physical quality of life (France, 42.98 vs. 45.91, p < 0.01; Germany, 41.97 vs. 44.11, p = 0.01) than the general population. Only trial participants in Germany had lower mental quality of life (47.38 vs. 49.74, p < 0.01) and significantly more emergency room visits and hospitalizations. Trial participants in France were less likely to have insurance than the general population (86.41% vs. 94.44%, p < 0.01). Clinical trial participants in the UK were more likely than the general UK population to report a preference for prescription vs. OTC medications (50.50 vs. 42.02, p = 0.02). CONCLUSION: Clinical trial participants are intrinsically different from the general population. This should be considered in the design and implementation of clinical trials and the generalization of results to the population.