METHODOLOGICAL ISSUES—Risk Assessment Studies

ADVANCING RISK ADJUSTMENT FOR SCHIZOPHRENIA

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OBJECTIVE: The objective of this study was to develop and validate a series of schizophrenia specific risk adjustment cost models. METHODS: Georgia Medicaid claims data linked with institutional inpatient data for 21,602 continuous eligible persons suffering from schizophrenia was used to build a prospective diagnosis-based, a demographic-based, a drug-based, and a combined risk adjustment cost model. ICD-9-CM and drug category classifications were derived from the literature and supplemented by an expert panel. Variables were screened and cost weights were derived empirically in a random 50% training sample using a robust a weighted Heuber-White regression model and validated by expert panel review, bootstrapping methods, and assessing indices of discrimination in a 50% validation sample. Model calibration and correlations of errors with policy relevant groups were also estimated. RESULTS: Measures of discrimination (R2) varied between 16.4% for the ICD-9-CM based model to 21.8% for the combined model for trimmed total cost and varied between 4.9% to 11.3% for mental health costs in the validation sample. Risk adjustment models based on drug or ICD-9-CM information discriminated costs equally well and the combined models outperformed both drug and ICD-9-CM based models. A simple model using prior year costs combined with demographic covariates had R2s > 40% for both mental health and total costs. CONCLUSIONS: The drug and ICD-9-CM based models performed equally well and either can be used with equal confidence depending on data availability. The combined models performed better than either the ICD-9-CM or drug based models indicating that drug exposure information can compliment more traditional approaches. Health services researchers wishing to control for differences in comorbidity and severity that influence cost should always consider including prior utilization (costs) since prior year costs were vastly superior predictors of costs.

OUTCOMES RESEARCH AND PHASE 1–2 PHARMACEUTICAL RISK ASSESSMENT

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OBJECTIVES: To determine the most effective way to determine risk benefit ratios in phase 1–2 developmental compounds. The EMEA and FDA have recently published discussion documents on the need to incorporate an assessment of risk benefit ratios during pharmaceutical development. While methods are established for the formal analysis of later stage development, the method to assess risk in early development is unclear. This paper seeks to identify what methods are available and assess their application to risk-benefit assessment in early stage products. METHODS: A literature review of the methods used to assess new technology risks. The nature of the risks involved in early stage (phase 1 and 2) pharmaceutical development were also identified from a database, and the core values of the risks determined by established frameworks. RESULTS: Four main methods were determined: formal analysis, bootstrapping, trade off and, judgement analysis. In the analysis of the nature of the risks of phase 1/2 pharmaceutical development much of the risk can be described as voluntary risk, due to the informed consent process. The acceptance of risk will therefore vary with the severity of the condition and the equity of the treatment being offered. The remainder of the risk is outcome related, and so needs to be viewed in the context of the aims of treatment. Comparing the methods used to assess risk, only Judgement Analysis was able to incorporate the degree of voluntary risk encountered in the risk assessment of pharmaceuticals in early development. CONCLUSIONS: The finding that Judgement Analysis is the only method to assess risk benefit in early stage development is controversial as it runs against the statistical methods favoured by the regulators. The results indicate a strong need to educate ethics committee and other clinical trial professionals in a wider range of outcomes research.

PROCESS UTILITY DERIVED FROM PROVIDING INFORMAL CARE

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OBJECTIVES: Though economics is usually outcome-oriented, it is often argued that processes matter as well. Utility is not only derived from outcomes, but also from the way this outcome is accomplished. Providing care on a voluntary basis may especially be associated with such process utility. In this paper we discuss the process utility from providing informal care. We test the hypothesis that informal caregivers derive utility not only from the outcome of informal care, i.e. that the patient is adequately cared for, but also from the process of providing informal care. METHODS: We measure process utility as the difference in utility between the current situation in which the care recipient is cared for by the caregiver and the hypothetical situation that someone else takes over the care tasks, all other things equal. We present empirical evidence of process utility on the basis of a large sample of Dutch caregivers (n = 950) and analyse these. RESULTS: Our results show that process utility exists.