include these effects and this appears not to influence results in general. More empirical work is needed, using generic instruments, larger and more relevant samples, and perhaps using the interview method of administration.

**PMC16**

**EXPENSIVE DRUGS FOR RARE DISORDERS AND THE LOGIC OF COST-EFFECTIVENESS**

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**OBJECTIVES:** Expensive drugs for rare disorders (EDRDs; “orphan drugs”) do not usually meet widely applied cost-effectiveness benchmarks (“lambdas”). Adopting the standard decision rules of the logic of cost-effectiveness cannot be reconciled with granting reimbursement status for many EDRDs and would inevitably deprive patients with very rare disorders from any chance to get access to effective treatment, given the high fixed / low variable cost structure of the pharmaceutical industry. On the other hand, public policies have been established to provide incentives to support development of orphan drugs. This (and some further observations) suggests a serious mismatch between the logic of cost-effectiveness and societal preferences. Decision-makers have responded; for instance, the National Institute for Health and Clinical Excellence (NICE) attempts to define a special subcategory of “ultra-orphans”—while maintaining that budgetary impact analysis is not part of its appraisal decisions (but limited to implementation support). This policy, however, does not appear to adequately address the underlying problem.

**METHODS:** First, “ultra-orphans” are not a distinct, well-defined category—they rather represent one extreme of a continuous spectrum, and “orphan drugs” and some cancer treatments pose the same fundamental problem. Second, size of a patient population eligible for treatment is directly linked to budgetary impact (and hence the opportunity for manufacturers to recoup fixed costs), whereas the logic of cost-effectiveness is impaired by not taking into account the size of the numerator and the denominator of the incremental cost-effectiveness ratio (ICER), which has been described as “the silence of the lambda.” Policy makers might address these issues by explicitly taking budgetary impact into account when deciding on maximum reimbursement prices or by price-volume agreements.

**RESULTS:** Both approaches, albeit perhaps pragmatic, cannot satisfy from a theoretical economic perspective. **CONCLUSIONS:** Rigorous normative analysis and empirical research are required to further explore the mapping of individual health-related utilities into societal preferences (willingness-to-pay).

**CONCEPTUAL PAPERS & RESEARCH ON METHODS—Databases & Management Methods**

**PMC17**

**VALIDITY OF ELECTRONIC PRESCRIPTION CLAIMS RECORDS: A COMPARISON OF ELECTRONIC PBM CLAIMS RECORDS WITH PHARMACY PROVIDER DERIVED RECORDS**

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**OBJECTIVES:** To determine if and to what extent records obtained from PBM pharmacy claims differ from source documents obtained directly from pharmacy providers. Also this study sought to explore possible associations between patient, pharmacy benefit, and pharmacy provider characteristics and the likelihood a patient would have missing prescription claims.

**METHODS:** This study used a cross sectional survey of 1,484 patients residing in a single state with a common pharmacy benefit. Patient profiles describing all prescriptions filled in a pharmacy between January 1, 2002 through June 30, 2002 of these patients were requested directly from their pharmacy providers. Logistic regression was used to explore the factors associated with a person receiving a prescription that did not appear on the PBM claims. **RESULTS:** Of the 1,484 eligible recipients sampled, profiles were obtained for 323 (22%) persons and there were analyzable profiles for 315 (21%) persons. Of those 2,977 prescriptions filled for the 315 persons, 207 (7.0%) were missing from the claims files indicating that 93% were captured. Prescription drugs such as iron products, digoxins, diuretics, sulfonylureas, and antigout were more likely to be missing from the PBM claims. Only prescription volume consistently influenced the likelihood a patient would have a missing prescription from the PBM claims (OR = 1.08; 95% CI: 1.05–1.12). **CONCLUSIONS:** Claims obtained from pharmacy benefit companies capture approximately 93% of prescription records when verified with records obtained from pharmacy providers. The rate of missing records from PBM claims does not appear to be meaningfully influenced by most finance based pharmacy benefit design features, however, certain drugs available over the counter and less expensive drugs may have less complete claims records compared to other classes of drugs. Higher prescription utilizers are more likely to have prescription records filled that are not captured by PBMs.

**CONCEPTUALISING DISEASE: BUILDING UNIFYING MODELS TO SUPPORT THE DEVELOPMENT OF PROS AND COST-EFFECTIVENESS ANALYSES. A CASE STUDY IN ALZHEIMER’S DISEASE (AD)**


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**OBJECTIVE:** The core of a cost effectiveness model should be guided by the underlying disease process, natural history and how particular interventions impact on disease. The development of conceptual models within PRO research is based on the same rationale. The objective of this study was to explore the overlap between cost effectiveness modelling and conceptual models for PROs, using AD as an example. **METHODS:** A conceptual model of AD was reviewed alongside a cost effectiveness model developed by a team of PRO researchers and health economists. Areas of commonality and divergence were documented and discussed. **RESULTS:** Points of overlap were identified which included the impact of AD on day to day functioning of the patient and associated caregiver burden. This will influence the ability of the caregiver to work and impact on other resource utilisation. This may, also have an impact on the time to institutionalisation, which is a major financial burden in AD. One major area of divergence is the emphasis in economic models on the use of generic quality of life data; whereas conceptual models are commonly developed to measure disease specific PRO burden. **CONCLUSIONS:** The commonalities between PRO conceptual models and health economic models indicates the potential for developing PRO and HE models simultaneously. This is likely to increase the validity of each of the models as well as having a positive impact on related research e.g. through the development of health state utilities. As a next step we are prospectively developing a conceptual model of disease which is specifically...