A BAYESIAN APPROACH TO PREDICT EFFECTIVENESS OF NEWLY INTRODUCED DRUGS IN DAILY PRACTICE BASED ON THE RELATION BETWEEN EFFICACY AND EFFECTIVENESS OF COMPETING INTERVENTIONS

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OBJECTIVES: Clinical trials required for licensing are designed to study efficacy and safety. Information on the effectiveness in routine practice, which is of interest for cost-effectiveness evaluations, is not usually available at the time of launch. As a result, efficacy data from clinical trials are often used as a proxy for effectiveness in cost-effectiveness evaluations. However, a difference between efficacy and effectiveness in the “real world” of practice can be expected for some drugs, due to patient selection, non-compliance, and treatment patterns. Therefore, how the efficacy of a new drug might translate into effectiveness in routine practice - both in terms of intermediate and hard clinical endpoints - is of primary interest for decision makers.

METHODS: A Bayesian Hierarchical model is introduced by which simultaneously a meta-analysis of direct and indirect comparisons is performed using efficacy and effectiveness data. At the first level of the hierarchical model a parametric model is set up for each individual efficacy and effectiveness studies; at the second level a parametric model is constructed to relate the parameters from separate efficacy studies to each other, as well as a model for the effectiveness studies. Intervention specific differences between efficacy and effectiveness are related to each other with a third level. The Bayesian approach can accommodate a priori beliefs regarding differences for the link between the efficacy and effectiveness across interventions. A particular appeal of the Bayesian approach lies in the prediction of the effectiveness of a new intervention, and calculation of the probability that this intervention is better than the current ones. RESULTS: The methodology is illustrated with analysis of the efficacy and effectiveness of statins in terms of LDL reductions and effectiveness across cardiovascular endpoints. CONCLUSIONS: The outlined approach can be used to predict effectiveness of new drugs awaiting higher quality evidence from randomized naturalistic trials.

ALLOCATION OF PRESCRIPTION DATA BY INDICATION: A METHODOLOGY INTEGRATING COMPLEMENTARY PATIENT-LEVEL INFORMATION SOURCES

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OBJECTIVES: Many recent FDA approvals have not been for new chemical entities, but for new indications for drugs already on the market. This creates new challenges for pharmaceutical companies and health plans to monitor use by indication in a timely manner, which may be necessary for monitoring compliance and disease management programs or managing prescription benefits by indication. Health plan claims data have the ability to link prescriptions to indication but typically suffer from a time lag. Longitudinal prescription data are available closer to real-time, but do not include indication. METHODS: We describe a modeling approach to parse out different indications for a product using a combination of health plan claims data and longitudinal prescription data. We explain how predictive models are developed, tested and validated, using these different data sources. Logistic models are developed using information from health plan claims data, representing the most robust source of medical and pharmacy claims linked at a de-identified patient level. Since the comprehensive health plan-sourced claims data may not be robust or timely enough for some applications, the data are used to develop a model that can be applied to more timely and robust longitudinal prescription information. RESULTS: The approach involves determination of appropriate diagnoses and drugs, construction of training and holdout data sets, development of logistic regression models, and evaluation of model performance. Electronic claims data could be used to provide diagnosis information, but patients’ claims may be missing, leading to gaps and inconsistencies in diagnosis-level reporting, causing the predictive models to be less robust and resulting in erroneous parsing of prescriptions by disease. CONCLUSIONS: The approach we propose is it makes the best use of disparate patient-level data sources, such as plan-based integrated claims data and pharmacy-based longitudinal prescription data to produce a robust and timely parsing of indication.