CRITICAL ASSESSMENT OF BELGIAN REIMBURSEMENT DOSSIERS OF ORPHAN DRUGS

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OBJECTIVES: This study aims to conduct an assessment of reimbursement dossiers of orphan drugs in Belgium. First, a qualitative analysis reviews the evidence submitted in reimbursement dossiers of all orphan drugs. Second, an in-depth analysis compares the clinical evidence submitted to the European Medicines Agency with the Belgian reimbursement dossiers for selected orphan drugs. METHODS: A qualitative analysis examined all reimbursement dossiers of orphan drugs that have been submitted in Belgium from January 2002 and June 2009. The following information was extracted from each dossier: description of the orphan drug; indication; reimbursement status; therapeutic value and needs; budget impact; and number of registered indications. For selected orphan drugs, an in-depth analysis extracted and compared information about the clinical trials, their primary endpoints and results from the following data sources: European Medicines Agency documents (i.e. the marketing authorization application file, the European Public Assessment Report and the Summary of Product Characteristics); and the Belgian reimbursement dossiers. RESULTS: Reimbursement was awarded to the majority of orphan drugs. In addition to the official criteria, other arguments such as the price, employment, patient population, funding of diagnostic tests by the company seemed to play a role in the reimbursement decision. Despite the low number of patients, randomized controlled trials were conducted for many orphan drugs. Budget impact analyses were simplistic and did not consider the impact across multiple indications. Also, some differences were observed in the clinical evidence submitted to the European Medicines Agency and the Belgian reimbursement authorities. CONCLUSIONS: There is substantial variation in the evidence that is submitted to the Belgian authorities in the context of an orphan drug reimbursement dossier. Also, some differences have been noted in the clinical evidence reported in European Medicines Agency documents and the evidence included in Belgian reimbursement dossiers of orphan drugs.