THE ROLE PLAYED BY SOCIAL COSTS IN ECONOMIC EVALUATIONS OF RARE DISEASE INTERVENTIONS

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OBJECTIVES: Although there is no consensus, the guidelines of the economic evaluation of healthcare interventions in several countries advise the use of a social perspective or a double perspective: social and health financing. This paper aimed to analyse whether the inclusion of social costs can affect the results in economic evaluations of rare disease interventions.

METHODS: A systematic literature review was designed to identify economic evaluations that included the healthcare perspective and the social perspective in the cost-effectiveness analysis from 2000 to November 2018. The inclusion criteria were: including an active substance considered as orphan drug by the EMEA or patients with a rare disease, being an economic evaluation of any intervention related to rare diseases, including informal care costs and/or productivity losses, being written in English, using QALYs as an outcome and separating the results according to the perspective applied. We launched this strategy on Medline and the Cost-Effectiveness Registry of the University of Tufts.

RESULTS: Social costs were included in 27 (10.8%) studies from 249 economic evaluation studies. However, we found only 14 manuscripts that fulfilled the inclusion criteria. The most of the studies (78%, n=11) assessed a pharmacological intervention. Productivity losses were included in 12 studies whereas informal care costs were included in 3. The method used for estimating productivity losses was human capital. The informal care costs were mainly assessed using the opportunity cost method. The differences in the results of incremental costs due to the application of a wider perspective changed the ICUR from values higher than zero to values lower than zero in 3 cost-utility analysis.

Figure 1: Flowchart of study identification and selection criteria

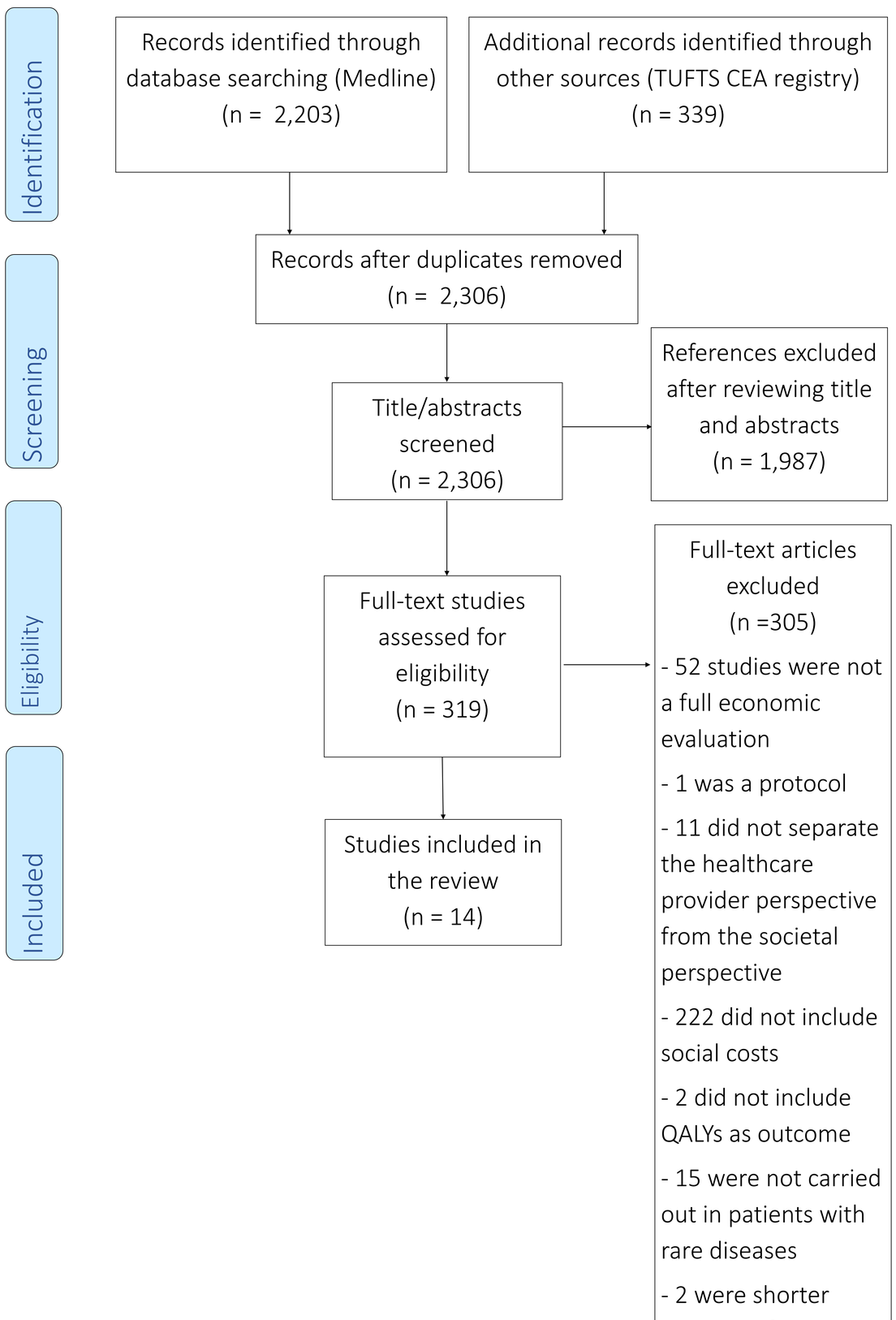


Figure 2: Incremental Cost-Utility Ratios (ICUR) in Rare Diseases treatments for low threshold values: Healthcare and Societal perspective.

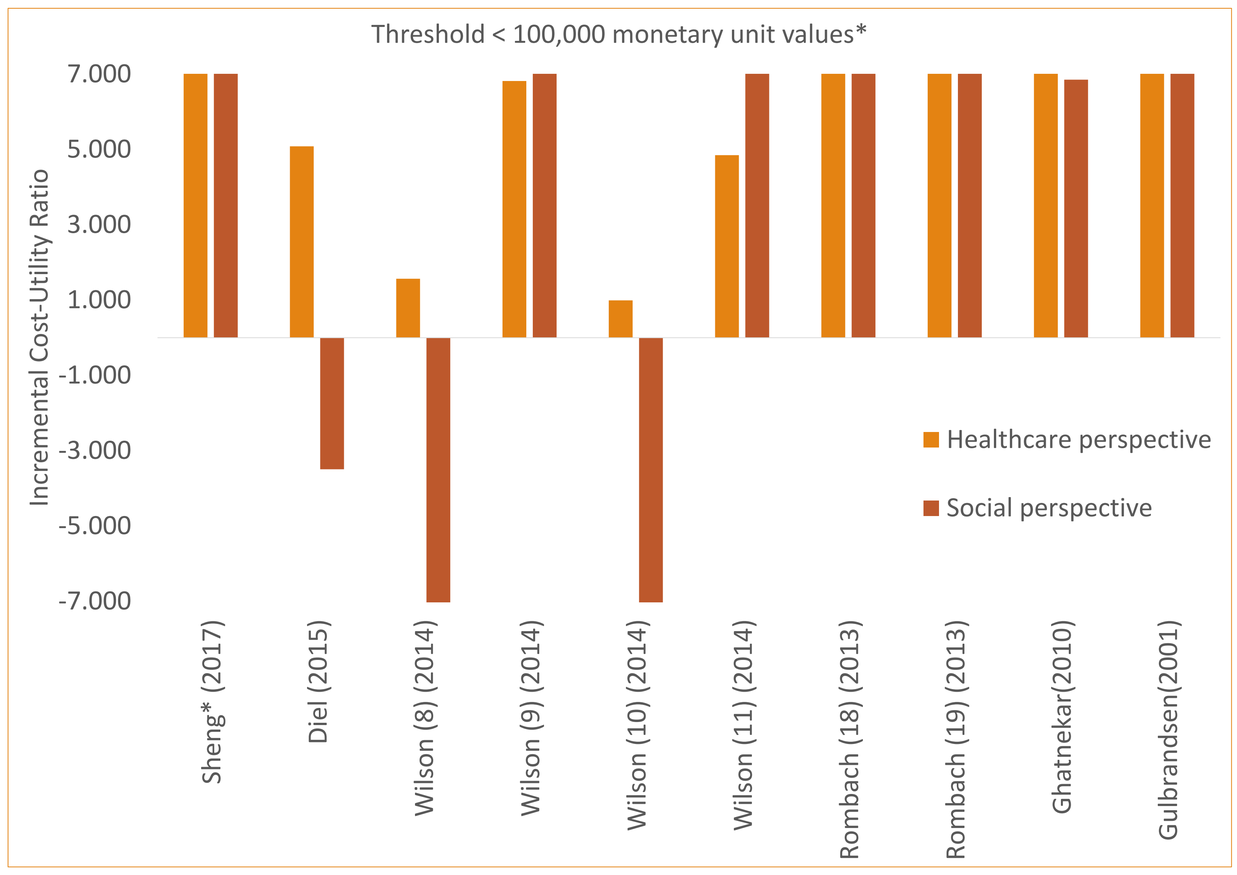
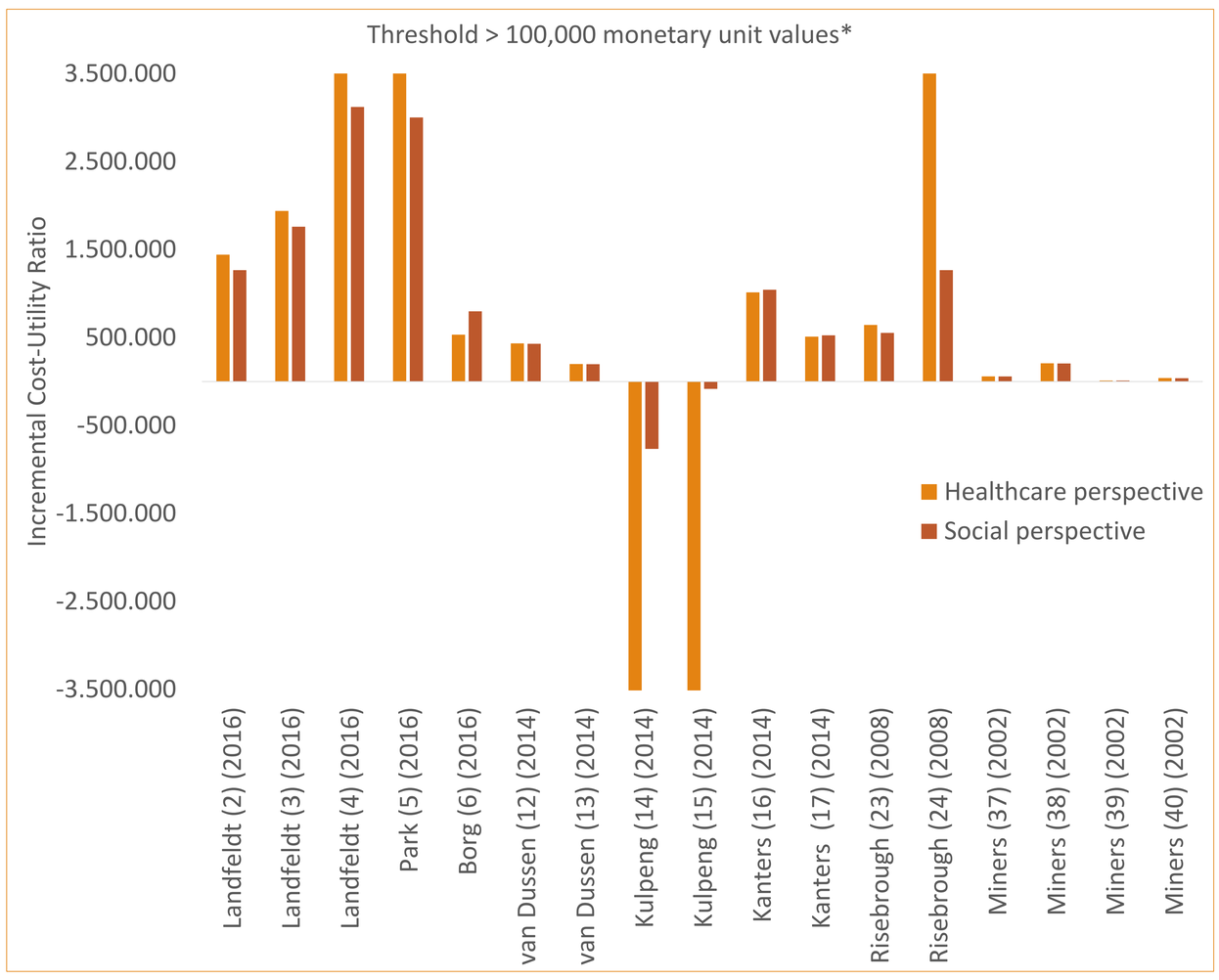


Figure 3: Incremental Cost-Utility Ratios (ICUR) in Rare Diseases treatments for low threshold values: Healthcare and Societal perspective.



CONCLUSIONS: The almost complete absence of the inclusion of the costs of informal care is surprising, despite its high relevance in rare diseases. The inclusion of social costs made the incremental cost-utility rate to become negative. However, it does not seem to influence the conclusions substantially.

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