

Special status for orphan drugs in health care decision making: a wise move?

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During the last 20 years drug licensing authorities in the developed world have established special procedures for treatments for rare diseases. The rationale for the special measures is that treatments for these rare diseases would not be forthcoming without them, and that it is inequitable that the 'quality of care' a patient receives should be affected by the number of people who have the disease. In addition, it has been argued that the development of such therapies is in the public interest. These measures have been extremely successful in promoting the development of treatments for rare diseases; e.g. The FDA has given orphan drug designation to over 900 therapies since the implementation of the US Orphan Drug act in 1984, although not all of these have reached the market.

In this paper we consider the potential economic arguments for the provision of special status to treatments for rare diseases. Specifically we examine arguments relating to the cost of production and those relating to the valuation of benefits. We then set out how the decision analysis framework, in which economic evaluation sits, takes appropriate account of disease prevalence. We conclude that there is no efficiency-based argument and that potential equity arguments which might apply, are not unique to rare diseases. Faced with the question 'Does rarity in its own right merit special measures?' we respond with an unequivocal 'No'. Finally, we set these conclusions in the wider context of developments in pharmacogenomics and proteonomics; illustrating how according special status for rare diseases to resource allocation decisions has the potential to bankrupt developed nations' health care systems.