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Health technology assessment and haemophilia.

Farrugia A, O'Mahony B, Cassar J.

Source

Plasma Protein Therapeutics Association, Annapolis, MD, USA. afarrugia@pptaglobal.org

Abstract

Although the funding of rare diseases such as haemophilia in developing countries remains a low priority, pressures on the funding of haemophilia treatment are also emerging in developed economies affected by the global economic downturn and the other demands on health care budgets. This is leading advisory bodies and payers alike to explore the tools of Health Technology Assessment (HTAs) in deriving recommendations for reimbursement policies. In particular, the use of cost utility analysis (CUA) in deriving costs per quality adjusted life year (QALY) for different interventions is being used to rank interventions in order of priorities relative to a threshold cost per QALY. In these exercises, rare chronic disorders such as haemophilia emerge as particularly unattractive propositions for reimbursement, as the accepted methodology of deriving a CUA. For e.g. the use of prophylaxis in haemophilia leads to a range of costs/QALY which exceed the willingness to pay thresholds of most payers. In this commentary, we review the principles utilized in a recent systematic review of the use of haemophilia products carried out in Sweden as part of an HTA. We suggest that ranking haemophilia related interventions with the standard interventions of therapeutics and public health in CUA comparisons is inappropriate. Given that haemophilia treatment is a form of blood replacement therapy, we propose that such comparisons should be made with the interventions of mainstream blood transfusion. We suggest that unequivocally effective treatments such as haemophilia therapies should be assessed differently from mainstream interventions, that new methodologies are required for these kinds of diseases and that evidence of a societal willingness to support people with rare disorders needs to be recognized when reimbursement policies are developed.