Expensive Drugs for Rare Disorders and the Logic of Cost-Effectiveness

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Expensive drugs for rare disorders (EDRDs; "orphan drugs") do not usually meet widely applied cost-effectiveness benchmarks ("lambdas"). Adopting the standard decision rules of the logic cost-effectiveness cannot be reconciled with granting reimbursement status for many EDRDs and would inevitably deprive patients with very rare disorders from any chance to get access to effective treatment, given the high fixed / low variable cost structure of the pharmaceutical industry. On the other hand, public policies have been established to provide incentives to support development of orphan drugs. This (and some further observations) suggests a serious mismatch between the logic of cost-effectiveness and societal preferences. Decision-makers have responded; for instance, the National Institute for Health and Clinical Excellence (NICE) attempts to define a special subcategory of "ultra-orphans" – while maintaining that budgetary impact analysis is not part of its appraisal decisions (but limited to implementation support). This policy, however, does not appear to adequately address the underlying problem. First, "ultra-orphans" are not a distinct, well-defined category – they rather represent one extreme of a continuous spectrum, and "orphan drugs" and some cancer treatments pose the same fundamental problem. Second, size of a patient population eligible for treatment is directly linked to budgetary impact (and hence the opportunity for manufacturers to recoup fixed costs), whereas the logic of cost-effectiveness is impaired by not taking into account the size of the numerator and the denominator of the incremental cost-effectiveness ratio (ICER), which has been described as "the silence of the lambda." Policy makers might address these issues by explicitly taking budgetary impact into account when deciding on maximum reimbursement prices or by price-volume agreements. Both approaches, albeit perhaps pragmatic, cannot satisfy from a theoretical economic perspective. Rigorous normative analysis and empirical research are required to further explore the mapping of individual health-related utilities into societal preferences (willingness-to-pay).

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