Editorials

Are drugs for rare diseases "essential"?

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In 1977, the first report of the WHO Expert Committee on the Use of Essential Drugs defined essential drugs as those needed to satisfy the health-care needs of the majority of the population. This was done in order to fulfil a mandate to assist Member States in selecting and obtaining essential medicaments for their populations. The Expert Committee then developed criteria for determining if a drug fitted this definition and published a Model List of Essential Drugs as an example of how the concept of essential drugs could be implemented.

A brief review of the state of medications at that time explains the need for this assistance: resources were limited in many countries, so the goal was to use them wisely, and many drugs marketed around the world were ineffective or irrational combination products. A review of the evidence of efficacy of all prescription drugs on the market in the United States starting in 1966 found that about one-third of the over 3000 marketed drugs were not effective.[1] In addition, there were frequently several effective drugs in the same therapeutic class: all did not need to be stocked by health service pharmacies. The essential drugs concept and the methods for its implementation were developed to help make decisions about which drugs to purchase to make the best use of the available health-care resources.

WHO reported that, by the end of 1999, 156 Member States had medicine lists indicating acceptance of the essential medicines concept. Furthermore, there are a number of published examples of the favourable impact of this concept on various regions in the world.[2] While the procedures used by this WHO activity have evolved over time,[3] the definition of an essential medicine continues to be a medicine needed for the majority of the population. This appears to exclude systematically medicines for rare diseases. Is it time to change the definition?

In this issue, Stolk et al. identify changes in the policies of some governments to facilitate the discovery and development of drugs for uncommon diseases. They propose an additional complementary Orphan Medicines Model List to include drugs for rare diseases in the Essential Medicines programme of WHO.[4] They propose seven criteria for including a drug in their suggested list; cost is not one of them. How should treatments for uncommon or rare diseases be considered, if at all, for an essential medicines list? After all, there are more than 6000 rare diseases and related conditions listed on the National Institutes of Health web site.[5]

Aristotle raised the principle of distributive justice, the proper distribution of benefits and burdens, to address this question.[6] Is it right for one patient to benefit from a health service and another patient to be ignored only because of the prevalence of their illnesses? Is there a better way to select which medicines to purchase than to purchase only those for common problems? Are patients with rare diseases irrelevant to the health-care needs of a population?

One way to make decisions about resource allocation is through cost–effectiveness analysis. This was discussed 30 years ago[7] as a way to help rationalize the allocation of limited medical resources to produce the greatest good for the greatest number of people. Briefly, cost–effectiveness analysis compares the total cost of an intervention to its effectiveness. The analysis then reviews the cost–effectiveness ratios of the various interventions competing for limited resources. Priorities can be set for those interventions that give the most effectiveness for their total costs. Techniques for doing formal cost–effectiveness analyses are quite complex but the principle is straightforward. One can consider applying the principle to some obvious situations in which a detailed formal analysis may not be needed for decision-making while technical competence in cost–effectiveness analysis is developed to help with more difficult decisions.

If the definition of an essential medicine is to be changed to include medicines needed for people with rare diseases, then the principle of distributive justice can be the moral basis for such a change and cost–effectiveness analysis can be the method used to select which medicines to include in the Model List. I doubt that having a separate complementary Orphan Medicines Model List would make these medicines available, since these drugs would not be identified as "essential" without qualification. If cost–effectiveness analysis indicates high priority for a drug for any disease, it should be considered "essential" and put on the Model List of Essential Medicines. Whether highly cost-effective medicines for rare diseases should be considered essential medicines is the immediate question to be answered. The principle of distributive justice suggests that the answer is yes. ∎

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