

Allocation of Resources for Diagnostic and Therapeutic Interventions in Rare Diseases

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Abstract

Background: The health system encounters limited financial resources in all countries. Resource allocation is one of the tasks of the health system. Prioritizing interventions is one of the strategies that can help health policymakers allocate financial resources. Rare diseases require more attention than other diseases due to their high cost and complex treatments. The countries use different policies to determine the effectiveness of interventions in the field of rare diseases. The purpose of this study is to refer to some policies in the field of allocating resources for rare diseases as well as to explain the importance of determining the threshold of cost-effectiveness for rare diseases in Iran.

Methods: This research is a review study. First, a study was conducted on how to prioritize health interventions in the world and the thresholds of cost-effectiveness in different countries. Articles related to the research topic were then searched through accessible databases in Iran such as SID, Google Scholar, and Medline. Finally, the obtained articles were screened and analyzed based on a thematic approach.

Results: The World Health Organization (WHO) has set a threshold for determining the cost-effectiveness of health system interventions that are determined and calculated based on the per capita GDP of each country. There are many differences between countries on policies related to the treatment of rare diseases, medicines, health care budgets, and patient access.

Conclusions: Due to the very high cost of treating rare diseases, it is impossible to use the threshold used for general disease interventions in rare diseases, and it is necessary to use a higher threshold for rare diseases. In addition to cost-effectiveness, budget, justice, feasibility, and other criteria that are considered important at the national level should be considered.

Keywords: Rare diseases, Health system, Resource allocation

1. Background

Undoubtedly, health is the right of all people, and this has been accepted by everyone, especially health policymakers, as an undeniable fact (1). Today, all countries face the fact that the health sector, like other sectors, has limited resources (2). All health care systems in the world face the problem of resource scarcity. The main issue is how resources are allocated in a way that is fair (3). Resources allocation means the distribution of goods and services to different people and programs. In the field of health, large resources allocation is made by the government at the national, provincial, and city levels. Mid-level allocations take place at the organizational level, for example, in different departments of a hospital. Micro-level allocation takes place at the individual level (4). There are various methods for resources allocation based on need, which can be considered a range from simple indicators to complex models. Each of these models has strengths and weaknesses and is designed according to the condi-

tions of each country. The most appropriate way to allocate resources is to be designed based on the needs of the health system of that country and, in addition to being simple and transparent, use indicators that are a good representative of the health needs of people in different geographical areas of the country and information about indicators that model is also reasonably available (5). One of the most challenging issues in all countries in prioritizing health, policy-making, and service management is related to rare and incurable diseases. As aspects of policy-making, organizational structure, how to provide financial resources, and the service control mechanism of this type of disease has always been discussed. Owing to the chronic nature of rare and incurable diseases, lack of definitive treatment, heavy costs, and the need for complex treatments, many health resources have always been dedicated to rare and incurable diseases. Therefore, policy-making and organizing health services for these



types of patients and allocating resources to them is important (6).

2. Objectives

The purpose of this study is to refer to some policies in the field of allocating resources for rare diseases as well as to explain the importance of determining the threshold of cost-effectiveness for rare diseases in Iran.

3. Methods

This research is a review study. This research has been done in three stages. First, a study was conducted on how to prioritize health interventions in the world and cost-effectiveness thresholds in different countries. In the second stage, articles related to the research topic were searched in the databases available in the country, such as SID, Google Scholar, and Medline. Inclusion criteria were articles related to prioritization and resources allocation for rare diseases, and other articles were excluded from the study. Finally, 14 articles were found. In the last stage, the obtained articles were screened based on thematic relevance and analyzed, and their results were published.

4. Results

4.1. Allocation of Resources in the Health System

The expansion of health care around the world and, as a result, an increase in costs impose an increasing financial burden on the health care systems. However, countries

are unable to provide all health services to patients due to limited financial resources (7). The result of resource constraints is that countries encounter choices. Hard decisions must be made when the gap between the need for health care and the number of available resources is widened (8). With these points in mind, it is important to make the most of the available resources. To ensure this is done, we need to prioritize and allocate resources optimally (9), but there is little consensus on choosing the most optimal tool (2). Health technology assessment is one of the tools that can help us in this way. Health technology assessment is a multidisciplinary field of science aimed at analyzing clinical, social, economic, and ethical issues related to the application of technology in the health system as a type of policy-related research (10).

4.2. Resource Allocation Tools in the Health System

Many countries are now using cost-effective analysis to guide their decisions about resource allocation and to compare the effectiveness of alternative health interventions. The cost-effectiveness threshold is set to identify cost-effective interventions. The World Health Organization (WHO) has set a threshold for cost-effectiveness that is determined and calculated based on the per capita GDP of each country. It is worth noting that the cost-effectiveness threshold is based on the outcome of QALY or the cost required to add one year of quality life to individuals, and the threshold in the health systems of different countries is dissimilar, some of which are mentioned below (11) (Table 1)

Table 1. Threshold to per Capita GDP (for all Health Interventions) and C/E Threshold in Some Countries

Country	Threshold to per Capita GDP (for all Health Interventions)	C/E Threshold
Australia	1.53	76,707
Poland	3	84,857
Thailand	0.8	13,569
England	0.66	28,833

There are criticisms of GDP-based thresholds because people value life beyond income (12). In other words, from the people's point of view, the value of life is not comparable to people's income. The information on which the cost-effectiveness threshold is based is almost entirely derived from the high-income countries of North America, Western Europe, and Australia (13). Decisions should not use the threshold of cost-effectiveness as the sole criterion for determining the price or cost of repaying a drug or a new intervention (14). Rather, in addition to cost-effectiveness, budget, justice, feasibility, and other criteria that are considered important at the national level should be considered (15).

4.3. Resources Allocation in Rare Diseases

Resource allocation is very important in evaluating technologies related to rare diseases, as these technologies,

such as drugs for these diseases, are rarely cost-effective due to their high cost and limited effectiveness, and lack of coverage of non-cost-effective technologies is often met with political and social resistance in societies (10). Rare diseases are unusual and serious cases defined in the European Union (EU) as a condition that threatens a person's life or leads to chronic disability, and the prevalence of such diseases is not more than 5 in 10,000 people (16). Between 6,000 and 8,000, rare diseases have been identified, most of which are of genetic origin and are associated with severe clinical manifestations (17). Rare diseases in Iran, which are determined based on available information, currently include 309 diseases (18). "Eighty percent of rare diseases have identified genetic origins, 50% of rare diseases affect children, and 30% of patients with rare diseases die before the age of 5" (19). "Rare diseases have often been neglected by pharmaceutical companies, and patients suffering from rare diseases thus have less ac-

cess to relevant therapy” (20).” Majority of orphan drugs authorized in the EU are not available in Latvia. Moreover those drugs that are available are often not accessible because they are insufficiently reimbursed” (21). In most cases, the cost of treating rare diseases is very high, and to get a year of quality life, one has to spend a lot more than three times per capita GDP, more than the increasing cost-effectiveness threshold announced by the WHO. In Iran, for example, the annual cost of treating lysosomal storage patients is 17 times per capita GDP. This demonstrates the need to design an efficient framework for how to allocate resources for the treatment of rare patients (22).

4.4. Policies of Different Countries in Rare Diseases

There are many differences between countries in terms of policies related to the treatment of rare diseases, drugs, health care budgets, and patient access. The cost-effectiveness threshold for rare diseases in the UK is different from other diseases. The process that is currently being considered by the UK’s National Institute for Health and Care Excellence (NICE) to evaluate rare disease technologies is to use the cost-effectiveness threshold of 100,000 pounds per QALY (3.3 per capita GDP), on the condition that their annual budget impact is less than 20 million pounds per year (in the first three years of clinical use) (23). “The NHS evaluates therapeutic value, price, expected budget impact, and cost-effectiveness for each drug before it is included in the reimbursement list. Drug price is compared with the prices in other EU countries. The price of the reimbursed medicine should not be higher than the third lowest price in the Czech Republic, Denmark, Romania, Slovakia and Hungary, and shall not exceed the price of the medicine in Estonia and Lithuania” (21). Some countries are looking for new ways to estimate the cost-effectiveness of rare disease drugs, including Poland, which has decided to use the multi-criteria decision analysis (MCDA) method in its rare disease policy (17). Treatment of rare diseases exceeds the standard cost per QALY. Multi-criteria decision analysis is an approach that can be used to consider other aspects of technology. This type of analysis, by weighing the factors that influence specific interventions, provides results that can be used to compare technologies (24, 25). The method of MCDA is defined in four stages. In the first stage, characteristics such as the prevalence of the disease, social and economic conditions, and budget are determined. In the second stage, each of the characteristics is given weight. In the third stage, each drug is evaluated based on its characteristics. Finally, in the last stage, the drugs are ranked, and the drugs that have the highest priority are negotiated by the stakeholders, whose negotiations can change the technical rankings slightly to include socio-economic conditions in this quantitative analysis.

5. Discussion

As global studies show, in some developed and develop-

ing countries such as the United Kingdom, Scotland, and Thailand, a certain threshold is used for economic evaluation studies of health interventions, and in Iran, a certain threshold related to per capita GDP is used in economic evaluation studies (26). However, due to the very high cost of treating rare diseases, it is impossible to use the threshold limit for general disease interventions in rare diseases, and in this regard, it is necessary to use a higher threshold, which is sometimes several times the cost of general cost-effectiveness. In the case of rare diseases, it seems necessary to conduct a separate study to extract the threshold for the willingness of society to pay for this type of patient.

In addition, the mere use of economic evaluation is not sufficient in health technology assessment studies for these patients, and like other health interventions, other aspects of therapeutic interventions for these patients should be considered, including the budgetary impact of interventions. In this approach, health technology assessment studies related to rare diseases go beyond the cost/QALY calculation framework and are evaluated in a comprehensive decision-making framework using MCDA, as well as negotiation and consensus of stakeholders. Based on the experiences of these countries in health technology assessment related to rare diseases, more extensive considerations are required about the nature and type of these diseases, their treatment experiences from the perspective of stakeholders, and technical measures to reduce the level of uncertainty about cost-effectiveness calculation. Some of the most important criteria of this framework are the social and economic conditions of each country, the cost of national health services and the budgetary impact of each intervention, the cost-effectiveness ratio, and the indirect effect of the intervention on the provision of public and specialized services and on other parts of the health system of that country (10, 27).

Therefore, in order to make a cost policy for the therapeutic interventions of rare diseases, it is first necessary to separate and determine the share of the budget allocated to these types of diseases from the total health resources, based on optimization calculations. The level of willingness to pay in the community for these patients should also be determined. Then, the pharmaceutical package and healthcare services of rare patients are reviewed, and based on the studies of health technology assessment and applying the principles of multi-criteria decision analysis, the final policy is made for the pharmaceutical package and the healthcare services of these patients.

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