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Cost-Effectiveness and Dynamic Efficiency: Does the Solution Lie Within?

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ABSTRACT

The majority of the current systems spread across the world require the value of pharmaceuticals to be demonstrated with an acceptable degree of certainty before a technology is funded. Often involving the notion of cost-effectiveness, one of the key characteristics of such assessments tends to be the consideration of efficiency as a static outcome; with a strong emphasis on current health gains but a disregard for the impact of decision making on the potential health value over time. In this article, we argue that current systems using cost-effectiveness thresholds may provide an incomplete indicator of value. We defend the idea that funding decisions should also be informed by dynamic efficiency considerations and reflect both the current and the future value of achieving a certain level of effectiveness in a specific disease area. We further lay down the foundations for the implementation of such a value assessment framework.

Keywords: cost-effectiveness, pharmaceutical innovation, dynamic efficiency, value framework.

Cost Effectiveness and a Static Notion of Efficiency

In publicly financed health care systems, policymakers operating under budget constraints and expenditure controls establish pharmaceutical pricing policies that attempt to balance short- and long-term health policy objectives. Although the process varies substantially across jurisdictions, it often involves two distinct phases [1]. In a first assessment phase, efficiency is considered mainly by quantifying the incremental clinical and economic benefit of a new technology. At this stage, extensive clinical trial evidence is gathered and new innovative health technologies are compared with existing alternatives both in terms of costs and clinical outcomes. In theory, a second appraisal phase will then consider other factors, such as equity or perceived clinical need, to evaluate the full societal value of a technology.

To be funded under this system, new technologies are required to represent an acceptable and/or affordable cost per unit of incremental health-related benefit over existing health care technologies. In this context, affordability and acceptability are defined by the efficiency of other available technologies, and the health budget constraints often take the form of a cost effectiveness threshold. This threshold is expected to represent the opportunity cost of displacing an old technology to fund a new one [2] and is usually applied universally across all diseases and conditions [3]; in some jurisdictions, however, the application of a higher or lower threshold during the appraisal stage may depend on elements such as the severity of illness. In essence, the current framework applies an extrawelfarist approach using cost-effectiveness thresholds in the estimation of the net benefit resulting from the introduction of a new technology, which will be then used as a decision rule.

Although the framework is theoretically sound from a resource allocation perspective, difficulties in proving value in the required time frame when high uncertainty exists may result both in expensive false positives (i.e., drugs that do not prove to be cost-effective once in the market after being approved) and in inefficient launch delays (i.e., drugs being wrongly labeled as cost-ineffective, resulting in the loss of efficient health benefit due to a lack of access until uncertainty is resolved).

The potential for cost-effectiveness of prospective candidates in drug development is increasingly being considered at earlier stages by the pharmaceutical industry when defining research and development (R&D) investment strategies. However, the main elements that affect that potential, such as market structure, the pricing pattern of available comparators, or the

Current cost-effectiveness threshold-based systems provide a static notion of value, but the proposed framework allows funding decisions to be informed by dynamic efficiency considerations.

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evolution of clinical effectiveness over time, vary significantly across disease areas. Furthermore, the existing cost-effectiveness framework for appraising drugs at launch appears to systematically overlook any dynamics expected across disease areas and the preferences of future generations in tackling different diseases.

Cost Effectiveness and Dynamic Efficiency

In economic terms, static efficiency relates to efficiency at a particular point in time and is embedded with the notion of the technical efficiency of the health system in achieving the best health outcome possible with the resources available. On the other hand, the concept of dynamic efficiency refers to resource allocation over time to achieve a stage where it is not possible to increase welfare by any reallocation across players in different time periods. In its essence, the pursuit of dynamic efficiency is concerned with the optimal rate of innovation and investment to improve production processes.

From a societal perspective, an economically efficient system would minimize the sum of static (related to restricted consumer access due to monopoly pricing or monopsony power [4]) and dynamic (related to shortness and misdirection of incentives for R&D investment) inefficiency. Innovation becomes inefficient when the discounted sum of the incremental monetized benefits of subsequent steps of innovation is smaller than the corresponding incremental costs. From a static perspective, investment should be made, providing the incremental net benefit for an individual step is positive. From a dynamic perspective, however, the impact of a step in allowing future developments should also be considered. Hence, in some cases it will be efficient to pursue innovation even if that implies funding a step that is considered inefficient when assessed individually.

The innovative contribution of a drug in the progress toward a cure may represent a gain in perpetuity for future generations. In theory, the health care budget, and consequently any cost-effectiveness threshold applied, should explicitly represent current and future preferences and, eventually, the budget expected in different time periods. Although it is often claimed that cost-effectiveness assessments consider the societal value brought by a particular health technology, in practice, these assessments tend to focus on the maximization of an aggregate output without necessarily considering how that output is distributed in time or form. By preventing the incorporation of societal preferences toward future innovation, current technology assessments will distort the incentives for medium- to long-term dynamic efficiency [5].

Given that the perceived value of a technology may also depend on the value society places on treating specific conditions or particular groups of patients, the notion of value should encompass the absolute amount of health benefit gained (as derived from health benefit–driven societal preferences) irrespective of where and when it is achieved (Fig. 1). Because the factors that determine the potential for cost-effectiveness are expected to vary across conditions [6,7], the trade-off (i.e., the amount of health forgone) between static decision rules and dynamic implications for future generations is not expected to be the same across disease areas.

It can then be said that the use of a universal and, essential static, cost-effectiveness threshold does not accommodate this perspective, preventing incentives from reflecting societal preferences toward future innovation [8]. As a consequence, the threshold may misdirect investment, resulting in the disruption of the innovation path and compressing the broader research spectrum toward what is expected to be deemed immediately cost-effective rather than what is wanted by society in the long term. This will inevitably result in a lack of innovation in key areas of need and, consequently, lower welfare for future generations [5]. A striking example of this situation is reflected in the lack of incentives to support investment in the development of new antibiotics.

A Novel Dynamic Framework

Discussions of potential reforms across the world appear now to be more receptive to this dynamic element [9]. In theory, there may be scope to incorporate explicitly the industrial aspect of bringing new health technologies onto the market. A solution within the current cost effectiveness assessment framework that has the ability to capture the externalities in the production of innovation would necessarily include an indication of the individual contribution of a particular discovery toward disease cure (Figure 2).

Under such a format, the notion of option value (i.e., accounting for the externality of invention number one that allows progression to invention number two and subsequently invention number three) [10] could be incorporated in the assessment, with benefits and costs originated by underlying technologies in the future being discounted and included in the primer technology's value. In addition, the framework should consider the level of scientific challenge presented because, in some cases, advancement may imply greater investment in R&D.

To estimate an option value, whether at local or global level, three dimensions need to be assessed: 1) the relative value to society in achieving a particular clinical-effectiveness level in the long term; 2) the relative value of future and current health gains; and 3) the probability of achieving that target after the contribution of the discovery for such advancement. Thus, the incremental dynamic value of a technology would be represented by:

$$V_d = \left[IE_d + (\Delta u_D{}^* IE_D \text{ max})^* wEq_D\right]^* L \qquad \text{(Eq.1)}$$

where V_d is the dynamic value of incremental benefit d; IE_D is the incremental effectiveness brought by drug d;Δu_D is the change in the probability of achieving E_D max, that is, cure or maximum risk reduction for disease D due to innovation d, which is a function of the stock of knowledge and future R&D investment; IE_D max is the maximum incremental effectiveness possible for disease D; wEq_D represents an eventual priority weight attached to treating a particular disease D based on considerations such as equity; and L represents the static cost-effectiveness threshold.

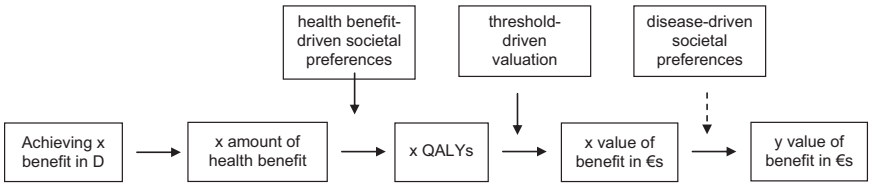

Figure 1 – Value of achieving an incremental clinical benefit in one particular disease area as assessed through current cost effectiveness-based assessment systems.