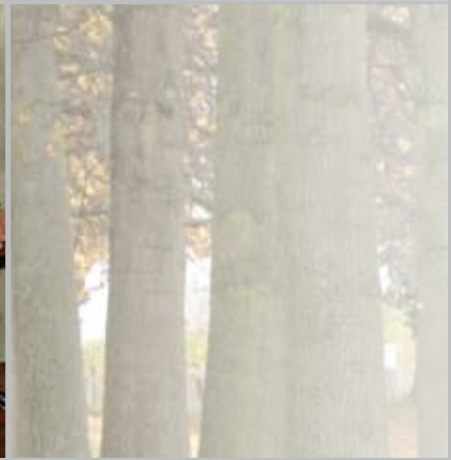


HEALTH TECHNOLOGY ASSESSMENTS

What Do They Mean for Plasma Protein Therapies?

BY JULIE BIRKOFER AND ALBERT FARRUGIA

HEALTH TECHNOLOGY ASSESSMENTS (HTAs) have joined the list of buzz words associated with health care reimbursement. Plasma protein therapies (PPTs) are not excluded from this involvement. It is therefore important to understand some of the basic principles around HTAs.



What are HTAs?

HTAs are a group of methods designed for evaluating health care interventions. They can be applied for examining the use of prevention programs, for using medical devices, for using drugs and for medical and surgical procedures. HTAs generally involve the use of *economic evaluation* – i.e., how much money is involved – and also draw on the *evidence* of the use of interventions.

Why the interest in HTAs?

As the population ages, the cost of health care increases – most health care is consumed by seniors. As medical technologies come into use, costs increase, prescribers and patients expect access to the latest innovations. The demands and expectations on health care are nearly infinite. At the same time, the capacity of payers (whether government or private) to reimburse consumers is under severe strain. Particularly during the current period of international economic downturn, methods are being sought to assist policy makers and payers to, effectively, establish ways of prioritizing interventions and, bluntly, ration health care and the allocation of scarce financial and human resources. *Inevitably, the use of HTAs leads to some patients receiving resources and treatments, and others not.*

Types of HTAs – Cost Effectiveness Analysis

HTAs involve the use of economic analysis and clinical evidence. One major HTA is *cost-effectiveness analysis* (CEA). In CEA, the costs of a treatment are determined and compared to the benefits as measured through some clinical outcome. It is the purpose of CEA to quantify the value of such costs and benefits. Examples of CEA in the field of plasma protein therapies would include the costs of avoiding a bleed in hemophilia when using prophylaxis, or the costs of immunoglobulin therapy to prevent infections in primary immunodeficiency (PID). The parameter resulting from the comparison of costs and benefits is called *the cost effectiveness ratio*. Since many CEAs are used to compare two interventions, this involves the generation and comparison of two cost effectiveness ratios, to assess the advisability or otherwise of choosing one intervention versus another. An example could be assessing the treatment of hemophilia using prophylaxis versus in-demand treatments, or comparing two different dosages for preventing infection in PID.

Cost – Utility Analysis and the QALY

Since CEA is widely used in policy making to develop priority scales for interventions, difficulties arise when widely different outcomes such as bleeds in hemophilia, infections in PID, respiratory volume in alpha-1-antitrypsin etc., come to be compared for their cost effectiveness. For this purpose, a special form of CEA has been developed, *Cost Utility Analysis* (CUA) in which costs are estimated for an intervention's ability to affect the overall quality of life of the patients involved. Rather than determine, for example, the cost of a treatment in terms of bleeds avoided or infections prevented, the cost of a year of the treatment's effect on the quality of the patient's life is calculated. To do this the *quality adjusted life year* (QALY) is

estimated. One QALY can be considered as a year in perfect health. For example, if the cost of prophylaxis in hemophilia is \$40,000 per QALY, it would mean that spending \$40,000 would result in one year of perfect health for the person with hemophilia. Similarly, if, again as an example, the QALY for a specific dose of immune globulin in PID is \$7,000 per QALY, it would mean that \$7,000 would result in one year of perfect health for a patient with PID. This allows all costs to be compared directly, given the commonality of the QALY.

HTA: Role in Decisionmaking

Since President Barack Obama signed the health care reform bill (ACA) into law last year, many provisions are intended to contain costs of federally funded programs while ensuring the delivery of quality health care. Two key provisions of the law in which policy-makers will rely upon to make decisions regarding patient access to plasma protein therapies are: comparative effectiveness research (CER) and the Independent Payment Advisory Board (IPAB). HTAs will likely play an important role in both and shape policies that impact patient access to plasma protein therapies.

As part of its section establishing an independent patient-centered CER body, the ACA includes a rare disease advisory panel,

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which U.S. stakeholders and PPTA championed. This provision requires the appointment of an expert advisory panel during each instance a rare disease is being considered for a CER study for the purpose of assisting in the design of the research study and determining the relative value and feasibility of conducting the research study.

While the CER provisions have strong safeguards for patient access, the IPAB provisions fail to protect patients in a similarly adequate manner. The purpose of the IPAB that will be created as a result of the passage of health care reform is to reduce the per capita rate of growth in Medicare spending. Beginning in 2014, the IPAB would submit to Congress and the Administration, any recommendations to this effect if the Chief Actuary of the Centers for Medicare and Medicaid Services (CMS) determines in the previous year that such a growth rate will increase faster than an established inflation rate. These recommendations would automatically go into effect the following year unless subsequent legislative action is taken by a certain date.

In this article, we have attempted to describe the basic principles of some HTAs. In future issues of *The Source*, we will continue to describe other HTAs and their direct application in plasma protein therapies.

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