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Pharmacoeconomics behind next-generation oncology drug development

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ABSTRACT: Molecularly targeted therapy is guided by an understanding of relevant genetic variations among individuals with a particular disease and, when applicable, by the relevant molecular variations in the expression of that disease. This approach has the potential to discriminate potential responders from nonresponders, identify which patients are likely to benefit earlier in the disease pathway, ensure appropriate dosing, reduce incidence of adverse events, and improve overall health gain. Stated otherwise, molecularly targeted therapy maximises the number of appropriately treated patients while minimising the number exposed to the treatment but in whom it ultimately fails. The benefits, however, must be balanced against the cost of screening tests to identify who is most likely to benefit from targeted therapy and against the lifetime costs of treatment.

Keyword: Pharmacoeconomics

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PHARMACOECONOMIC CHALLENGES OF MOLECULAR TARGETED THERAPY

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It is obvious that the status of pharmacoeconomics is now at a critical point. The challenge to health care payors is that they are confronted by the basic economic principle of resource scarcity and infinite demands. Health care resources are limited and pressure is increasing to contain the growth of overall health care

costs. Demand is driven by a combination of demographic change, patient knowledge and new technologies. Patient demand fuels the search for new technologies and increases pressure on the reimbursement systems.

Consequently, economic theory is increasingly being applied in health care decisions, specifically in regard to coverage for new technologies. Currently, a large proportion of lifetime health care expenditures occur at the end of life. Targeted therapies can shift the economics from excessive end-of-life spending to investing in prevention, earlier diagnosis, and early treatment of chronic conditions, Fig. 1. The early stages of developing molecularly targeted agents are likely to entail substantial investments in diagnostics and prevention, but savings might accrue during the later stages when diseases are prevented and/or treatments are applied with greater efficiency.¹

HEALTH TECHNOLOGY ASSESSMENT: Health technology assessment (HTA) is a multidisciplinary process that summarises information about the medical, social, economic and ethical concerns related to the use of a health technology in a systematic, transparent, unbiased and robust manner. Its aim is to inform safe and effective health policies that are patient focused and seek to achieve best value.²

Payors, whether they are governments or insurance companies, must be accountable to the taxpayers or policyholders who want to know that their contributions are used in an efficient manner. However, even the most robust HTA is subject to uncertainty from several sources, for example, the relationship between surrogate endpoints and final outcomes, the relationship between efficacy and effectiveness and resource use distri-

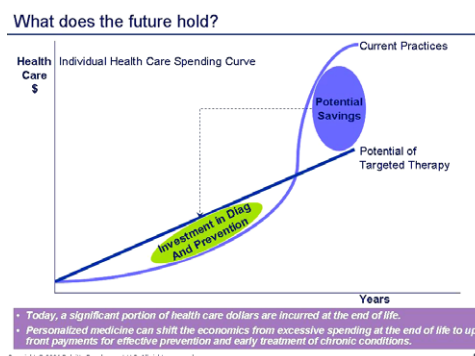


Fig. 1 – Savings from use of molecularly targeted agents could be invested in diagnostics development and cancer prevention.

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butions (e.g. hospital stays). Therefore, estimates of uncertainty must be factored into economic facets of HTAs.

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THE INDUSTRY-PAYER CHALLENGE OF NEXT-GENERATION ONCOLOGY DRUGS

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Industry, too, faces challenges as we enter the era of molecularly targeted therapy. Investment in the research and development can be significant, but a targeted therapy may be appropriate for only a subset of patients who have the correct molecular target. Screening would eliminate patients for whom the treatment would not be effective. As such, the development costs may increase but the potential patient population may decrease. Therefore, incentives need to be in place to ensure that manufacturers can realise a return on their investment.

Molecularly targeted therapy appears to offer an attractive value proposition; however, this can only be realised if industrial incentives are aligned with health care incentives. Introducing these therapies into practice presents some challenges. 'Both strong intellectual property protections and value-based, flexible pricing systems will be important in making personalised medicine a reality.'²

Health care expenditures, both in absolute terms and as a percentage of gross domestic product (GDP) are growing around the world. Innovative drugs are becoming more difficult to find, and more expensive to develop. Together these present significant challenges to both Payers and the Pharmaceutical Industry.

The challenge is developing successful next-generation oncology drugs within this environment. Success may be defined in several different ways. Success, to the patient, means access to a treatment that works. For the physician, it means using the right drugs in the right patients at the right time. Success, as defined by payers, connotes affordability and value for monies spent. Pharmaceutical companies seek success in the form of payback on their investment in research and development.

According to the traditional view of drug development and licensure, the product has three hurdles to negotiate: safety, efficacy and quality. In reality, however, at least three additional hur-

dles must be surmounted: national pricing and reimbursement, local/regional market access and health technology assessment (HTA). These last three include financial pressures in their evaluation. There is one additional challenge that must be considered at the outset of the development process – the need to measure value. To achieve success, pharmaceutical companies have to demonstrate that a product will deliver value (to the patient, and to the health economy) and net a return on their investment.

The highest hurdle is HTA, which has been defined as 'a multidisciplinary process that summarises information about the medical, social, economic and ethical issues related to the use of a health technology in a systematic, transparent, unbiased, robust manner. Its aim is to inform the formulation of safe, effective, health policies that are patient focused and seek to achieve best value.'³ Nearly 50% of licensed therapies fail to fully surmount the HTA hurdle in some way.

Not all drugs need to be subjected to the same levels of rigorous evaluation. The amount and quality of evidence required for licensure varies, and evidence required to support drug pricing and market access follows a parallel track. For example, therapies that are initially innovative (with price based on the value delivered) are eventually joined in the market by other drugs that are therapeutically similar, resulting in cluster-based or reference pricing.

Payer-Industry partnerships could be an attractive, and potentially successful approach in the future, as well (Fig. 1). Much might be gained by leveraging complementary skills sets and through access to, and analysis of, comprehensive (real-world) treatment and outcomes data. Ultimately, payers and pharmaceutical firms are working for the same person – the patient.

SURMOUNTING THE HTA HURDLE: HTA poses a number of questions regarding new therapies. First, how is the new treatment or technology to be used? This line of questioning should include the potential role or position of the therapy, the patients most likely to benefit from it, when in the disease course should it be used, and for how long. In addition to clinical-efficacy and – effectiveness, questions of cost effectiveness and resource utilisation must be asked: How much does the therapy cost? Is it affordable? Does it represent acceptable 'value for money'? (i.e. is it cost effective?) What is the best way to allocate scarce resources? Evaluation of cost effectiveness is often one of the most important components of HTA.

Key concepts often addressed are affordability, value for money, and willingness to pay. Implicit are issues of rational

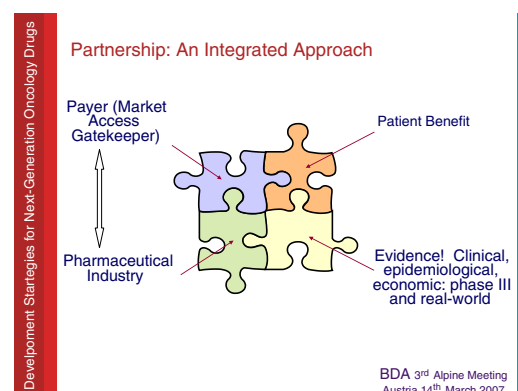


Fig. 1 – Payer-industry partnership: an integrated approach.