VALUE IN HEALTH 16 (2013) 901–906

Available online at www.sciencedirect.com

ScienceDirect

journal homepage: www.elsevier.com/locate/jval

POLICY PERSPECTIVE

Deciding Which Drugs Get Onto the Formulary: A Value-Based Approach

Raymond J. Seigfried, MA[1,*], *Teresa Corbo, PharmD, BCPS, FASHP*[1], *Mitchell T. Saltzberg, MD, FACC, FAHA*[1], *Jeffrey Reitz, PharmD, MPH*[2], *Dean A. Bennett, RPh, CPHQ*[1]

[1]*Christiana Care Health System, Wilmington, DE, USA;* [2]*Value Institute, Christiana Care, Newark, DE, USA*

ABSTRACT

Objectives: Hospitals, physicians, payers, and patients face economic and ethical decisions about the use of biotechnology drugs, commonly called specialty medications. These often target a small population, have data based on smaller clinical trials, are expensive, and may have questionable advantage. This is a result of how the Food and Drug Administration (FDA) approves medications, which is based only on safety and efficacy. Cancer drugs, once approved by the FDA, regardless of cost or value must be covered by Medicare. Some states have laws requiring additional coverage as well. All of this has created an unintended consequence: It has driven up costs with questionable evidence to support the medication's value, placing patients, payers, and providers in an ethical conflict. In this new era of health care transformation, health care leaders must focus on creating value to support a sustainable health system. Christiana Care Health System's Value Institute has designed a new model to evaluate specialty medications, using value as its main criterion. **Methods:** This article describes the process and outcomes using a new value model for evaluating specialty medications for a hospital formulary. It also introduces a new criterion of evaluation entitled "Societal Benefit" that provides a rating on quality- of-life issues. With measurable factors of efficacy, risk, cost, and quality-of-life concerns, our methodology provides a more balanced approach in the evaluation of specialty medications. **Results:** Specialty medications are the fastest growing segment of drug expense, and it is hard to understand how these medications will be sustainable under health care reforms. Unlike other countries, the United States has no national agency providing cost-effectiveness review; review occurs, if at all, at a local level. Laws governing Medicare and most private insurers' coverage of FDA-approved medication and some clinical quality standards conflict with cost-effectiveness, making this type of review difficult. Finally, because these medications affect the health system as a whole, it is a great example to begin to support health care reform. **Conclusions:** Hospitals need to challenge the value of specialty medication. Although our model will continue to evolve, value is now our central consideration when selecting specialty medications to be added to the formulary. We share this experience to encourage other hospitals to design their own approach to this vital issue.

Keywords: cost, efficacy, risk, societal benefit, value-based.

Background

Recently, a new category of medications called specialty drugs has emerged. These are very different from their forebears, both in effectiveness and in cost. They are high tech and high cost and are targeted at a very small percentage of patients, particularly in oncology, and they account for a much larger share of the overall cost. While many specialty drugs might slow the rate of disease or alleviate symptoms, few cure the disease long-term. Moreover, they can have severe or even fatal adverse effects and many have so-called black-box warnings from the Food and Drug Administration (FDA), or have manufacturer controls. Most of these medications are considered expensive, with total treatment costs sometimes exceeding $100,000 (with off-label use even higher) per patient.

The health care system has reacted to specialty drugs in a very predictable way. Insurance companies respond by passing on higher co-pays to the consumer. Twenty state governments react to this public concern by introducing or passing legislation limiting out-of-pocket payments for specialty medications. Insurance companies fear that by reducing payment by users, they shift the burden of increasing rates for everyone else. Physicians are confronted with quality standards that recommend using specialty medications regardless of cost. Hospitals shoulder the additional cost for these medications.

With ever more specialty drugs entering the market, it is increasingly important for hospitals and specifically their Pharmacy & Therapeutics (P&T) committees to develop an evidence-based and societal value approach to selecting which of these products should get onto the formulary. It might be argued that hospital-based reviews are not needed because the FDA has already approved the medication. In light of health care reform, hospital systems have no choice but to select drugs that are effective and have value.

Value Model

Christiana Care has assessed 12 of these medications by using a new value process. The mix of medications (oncology,

*Address correspondence to: Raymond J. Seigfried, Christiana Care Health System, 4755 Ogletown-Stanton Road, Newark, DE 19718, USA. E-mail: RSeigfried@Christianacare.org.

1098-3015/$36.00 – see front matter Copyright © 2013, International Society for Pharmacoeconomics and Outcomes Research (ISPOR). Published by Elsevier Inc.

http://dx.doi.org/10.1016/j.jval.2013.03.1623

pulmonary, rheumatologic, and immunosuppressive) allowed us to refine the process. Seven were approved by our P&T Committee and four were not. A final decision on one medication was deferred until a therapeutic evaluation is completed. Had the health system added all 12 medications to formulary, the annual projected cost impact would have been greater than $21 million.

Our traditional method of evaluating a medication assessed efficacy, safety, and cost. A clinical pharmacist developed a monograph summarizing the available literature and provided an estimate of annual cost based solely on acquisition cost and a projection of the number of patients likely to utilize the medication within a year. The monograph, which concluded with the clinical pharmacist's recommendation, was then reviewed by a physician who provided additional comments. The P&T Committee then decided whether the medication would or would not be added to the health system formulary. This process was reasonably effective until about 2010, when several newer medications reached the market and commanded considerably higher prices. The rising costs led us to question whether our formulary process was adequate or whether summarizing the available literature without a framework for assessing value could lead to rising costs with little or no improved outcomes.

We therefore formed a task force to establish a "value framework." It was led by the Administrator member on P&T with representative members from the medical- dental staff, pharmacy, nursing, and finance. After reviewing the literature and existing structures for grading levels of evidence, the four criteria we selected were efficacy, risk, cost, and societal benefit. Each medication would be reviewed against an equivalent medication or alternative therapy. In the event none existed, the medication would stand on its own merits. With the criteria determined, the P&T Committee established a Medication Value Subcommittee chaired by a physician and with members from administration, pharmacy, nursing, and finance.

The subcommittee decided upon a scorecard format. Assigning numerical values to the criteria allowed us to stratify variables among medications. This is especially useful when there are alternative medications to compare. While scoring a medication across the four criteria allows for easier comparison of therapies, we did not want to predetermine the P&T Committee's final decision. The scores merely inform the committee's final decision.

A physician reviews the monograph and, as with our traditional process, provides comments. The scoring and supporting information for efficacy, risk, and cost are presented to our Societal Benefit Team, which adds the score and commentary for that criterion. With the scorecard for all four criteria complete, the enhanced monograph is considered and discussed by the Medication Value Subcommittee. The subcommittee in turn makes a formal recommendation to the P&T Committee, which then makes the final decision on the medication's formulary status.

The P&T Committee is a standing hospital committee with 25 members representing our medical-dental staff, major specialties, pharmacy, nursing, finance, administration, and risk management. Over the last several years, the membership has had little turnover and has developed an effective interprofessional team approach in its review, dialogue, and decision making. Members speak openly and honestly during deliberation and the leadership of the committee chair, a physician, has been invaluable to this cohesion.

Efficacy

The efficacy portion of the value assessment has four domains: the outcomes reported in published studies, medication tolerability, the level of evidence for the observed outcomes, and the duration of the reported outcomes (Table 1). The P&T Committee decided to give efficacy the most weight in assessing medication value. This is consistent with the FDA decision to approve a medication on the basis of a favorable benefit-to-risk assessment, and avoids cost being given undue weight in the evaluation.

The categories of patient important outcomes are preventing disease, curing disease, improving ability to function by slowing disease progression or alleviating disease symptoms, and symptom palliation at the end of life. These outcomes are ranked and scored empirically. Medications that prevent or cure disease are given the most weight. Ranking last are those medications that relieve symptoms without improving ability to function, but this does not suggest that the positive effect that symptom relief has on quality of life is not valued.

After initial experience with the assessment process, we decided to include a qualitative measure of a medication's tolerability. Tolerability is evaluated in relation to the medication to which it is being compared. When no comparison is available, the medication is considered tolerable if fewer than 10% of the patients discontinued treatment because of adverse events. Medications less tolerable than the comparator or that were discontinued because of adverse effects by greater than or equal to 10% of the patients are scored lower than better-tolerated medications, as explained in the table. Tolerability is incorporated into the assessment process as a weighting factor. We recognized that a medication that is not tolerated well might not be preferred to an alternative, better-tolerated treatment.

One of the principles of evidence-based medicine is that there is a hierarchy of evidence that guides decisions about treatments [1]. At the top of the hierarchy is the randomized, double blind, placebo controlled trial, followed by systematic reviews of randomized trials and single randomized trials; at the bottom are unsystematic clinical observations. In between are observational studies and clinical studies that report surrogate outcomes. Stronger inferences about the efficacy of a treatment

Table 1 – Efficacy assessment.

Components of efficacy assessment	Score (points)
Patient important outcome	
Prevention or cure of disease	3
Improve function (slow disease, alleviate symptoms)	2
End-of-life care	1
Medication tolerability	
Tolerable	
Evaluated medication as tolerable or more tolerable than comparator	2
Incidence of discontinuation of evaluated medication <10% (no comparator)	2
Low tolerability	
Evaluated medication less tolerable than comparator	0.5
Incidence of discontinuation of evaluated medication ≥10% (no comparator)	0.5
Level of evidence	
Randomized trials assessing patient important outcome	3
Well done observational studies assessing patient important outcome	2
Other observational studies, expert opinion, unsystematic observations	1
Outcome duration	
>3 y	3
1–3 y	2
3 mo to <1 y	1
<3 mo	0