

The Promise of Specialty Pharmaceuticals: Are They Worth the Price?

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ABSTRACT

BACKGROUND: Specialty pharmaceuticals have evolved beyond their status as niche drugs designed to treat rare conditions and are now poised to become the standard of care in a wide variety of common chronic illnesses. Due in part to the cost of these therapies, payers are increasingly demanding evidence of their value. Determining the value of these medications is hampered by a lack of robust pharmacoeconomic data.

OBJECTIVE: To outline emerging strategies and case study examples for the medical and pharmacy benefits management of specialty pharmaceuticals.

SUMMARY: The promise of specialty pharmaceuticals: increased life expectancy, improved quality of life, enhanced workplace productivity, decreased burden of disease, and reduced health care spending—comes at a significant cost. These agents require special handling, administration, patient education, clinical support, and risk mitigation. Additionally, specialty drugs require distribution systems that ensure appropriate patient selection and data collection. With the specialty pharmaceutical pipeline overflowing with new medicines and an aging population increasingly relying on these novel treatments to treat common diseases, the challenge of managing the costs associated with these agents can be daunting. Aided by sophisticated pharmacoeconomic models to assess value, the cost impacts of these specialty drugs can be appropriately controlled.

CONCLUSION: Current evidence suggests that when used in targeted patient populations, specialty pharmaceuticals may represent a good health care value.

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Introduction

Specialty pharmaceuticals hold great promise for people living with an increasing number of chronic diseases. Accompanied by advances in genetic-based diagnostic techniques, specialty drugs also hold the potential to redefine the way illnesses are treated. As the combined use of these novel technologies are incorporated into routine clinical practice, pharmaceutical care will become more personalized. In the near future, physicians will prescribe drugs and select dosages that are tailored for each patient. The first steps toward personalized health care are visible today. Drug therapies designed for patients with specific genetic variations are already available (e.g., trastuzumab for HER2/neu expressing breast cancer), as are coordinated care programs for groups of patients with similar needs (e.g., those offered by manufacturers of specialty products). As the ability to practice personalized medicine evolves, it offers the potential for improving long-term outcomes and reducing cost of care across the spectrum of diseases.

The promise of specialty pharmaceuticals, however, is not without cost. Historically, specialty pharmaceuticals were developed for rare conditions affecting only a limited number of patients (e.g., Gaucher's disease). Because of their uniqueness, these agents often require special handling, administration, patient education, and clinical support—all factors that add to their cost. Despite being more expensive than traditional agents, they were viewed as a good value because they provided therapeutic options to patients afflicted with various cancers, hemophilia, and primary immune diseases who had few (if any) other choices. Consequently, their expense did not preclude their usage. Now, as use of specialty drugs rises faster than that of traditional therapies,¹ payers must scrutinize the costs associated with these agents, and payers are increasingly demanding evidence of their value.²

Growth of Specialty Pharmaceuticals

Specialty drugs have emerged as effective tools in treating a wide variety of illnesses. Expenditures on these agents are projected to increase from approximately \$54 billion today to more than \$99 billion in 2010.¹ It is estimated that specialty pharmaceuticals currently account for approximately 24% of total drug expenditures, but spending on these agents is rising about twice as fast as that for conventional drugs; a trend that is expected to continue for the next 20 years.¹ By 2030, it is anticipated that specialty pharmaceuticals will account for up to 44% of a plan's total drug expenditures.¹ A major factor in this growth is the large number of approved and soon-to-be approved specialty medicines. Since 1990, the number of approved specialty products has more than doubled every 5 years.¹ Today, nearly 200 specialty medicines have been approved by the U.S. Food and Drug Administration

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(FDA), and nearly 1,000 more are in development.¹ Today, growth in the specialty sector is driven by several chronic conditions, including cancer, rheumatoid arthritis (RA), and multiple sclerosis (MS).³

Unique Features of Specialty Pharmaceuticals

Specialty pharmaceuticals have several unique features that differentiate them from conventional drugs. These agents are typically administered by injection or infusion; processes that are more complicated and expensive than simply taking an oral dosage form. Because of their novel means of administration, specialty drugs historically have been reimbursed under the private payer's medical benefit or Medicare Part B rather than the pharmaceutical budget. Also, handling requirements for specialty drugs also are more complicated with many requiring refrigeration, special mixing or compounding, or concurrent lab work. Specialty pharmaceuticals are "high touch therapies," meaning that many patients require close monitoring and support during, and possibly for several hours following, administration. There is also a need for more intensive patient education and careful coordination of care between prescribers, payers, patients, pharmacy providers, and the administration site.

Because some specialty drugs are associated with an increased risk of clinically important or unusual and potentially harmful adverse effects, these agents can require increased safety surveillance. For products known to have the highest risk, the FDA mandates the implementation of a risk minimization action plan (RiskMAP), which is a strategic risk assessment program designed to minimize known risks of a product while preserving its benefits.⁴ A RiskMAP targets 1 or more safety-related health outcomes and uses 1 or more tools to reduce risk and related complications. It is an iterative process of assessing a product's benefit-risk balance, developing and implementing tools to minimize its risks while preserving its benefits, evaluating tool effectiveness, and reassessing the benefit-risk balance and making adjustments, as appropriate, to the risk minimization tools to further improve the benefit-risk balance.⁴ The FDA envisions this 4-part process to be continuous throughout the product lifecycle. In practice, the RiskMAP will require vigorous postmarketing monitoring and reporting of safety data by prescribers of specialty agents and by the distribution systems supplying them to patients, including specialty pharmacies.

Most specialty products will not need formal RiskMAPs, as routine risk minimization measures, such as appropriate labeling, are sufficient to ensure the safe and effective use of a drug or biologic product.⁴ The decision to implement a RiskMAP is made on a case-by-case basis and can be somewhat subjective. Data collected during clinical development, postmarketing surveillance, and Phase IV studies are used to inform a RiskMAP decision. In addition, an understanding of the nature and rate of known risks versus known benefits, the preventability of adverse events, and the probability of benefit in the product's target population

is required. As information about a product develops through its lifecycle, new data could direct a sponsor to properly determine if a RiskMAP is necessary where one was previously considered unnecessary.

The FDA provides a list of risk minimization tools, such as targeted education and outreach, reminder systems, and performance-linked systems, to achieve the objectives spelled out in the RiskMAP.⁴ However, the Agency provides minimal guidance on the processes by which these tools should be implemented. Currently, specialty pharmacies and other organizations involved in the distribution of specialty drugs are critically positioned to provide patient education and outreach, reminders, and collect data on performance. Thus, it is reasonable for payers to consider using the specialty distribution system to implement and manage FDA-mandated risk management programs. As a result, the RiskMAP program has the potential to increase the size of the specialty drug category, as medications not typically considered as specialty drugs will be classified as "specialty" because of risk mitigation requirements.

Categories of Specialty Pharmaceuticals

Specialty drugs are typically placed in 1 of 3 general categories: (1) self-administered therapies, such as those for RA, psoriasis, and MS; (2) products injected or infused in a clinic or office setting, including vaccines and treatments for various immune disorders, asthma, or hypogammaglobulemia; and (3) office/clinic administered chemotherapeutic agents (Figure 1). At first glance, it may appear that vaccines should not be categorized as specialty products because these agents are traditionally derived from viral or bacterial cultures and are available at relatively low cost. However, specialty vaccines are emerging as potential agents for the primary prevention of chronic disease. For example, a vaccine for the prevention of human papillomavirus was licensed for use by the FDA in 2006, and a second vaccine is expected on the market soon.⁵ Likewise, a vaccine to prevent shingles in people aged ≥ 60 years was recently approved.⁶ In addition, immunizations are currently under development for Alzheimer's disease, MS, human immunodeficiency virus, various forms of cancer, and other conditions.⁷ These emerging vaccines will share several features more in common with today's specialty agents than with traditional vaccines, including the processes used to develop and manufacture the vaccine and product price.

Coordinating Access and Distribution

Because of their complexity, specialty pharmaceuticals flow through a variety of distribution channels. These channels vary widely according to the specialty product's administration requirements, the payer's benefit design, and the provider's service availability. In addition, manufacturers may control the distribution of specialty products through selected distributors due to limited production capacity and special handling requirements.

Channel selection not only affects the efficiency by which a drug is delivered to a patient, but can also impact the outcomes experienced by patients. Channel selection may also have ethical consequences. Payers must determine if the health plans are in the best position to coordinate access and delivery of specialty products. If plans are not capable of doing so, specialty pharmacy services might be considered. Given what we know about the conflict of interest inherent in provider-centric payment mechanisms, such as “buy-and-bill,” another important consideration is if prescribers are capable of selecting the most appropriate treatments without undue bias. Finally, it must be determined if high copayments or coinsurance limit access to appropriate specialty therapy and, therefore, compromise clinical and safety outcomes.⁸

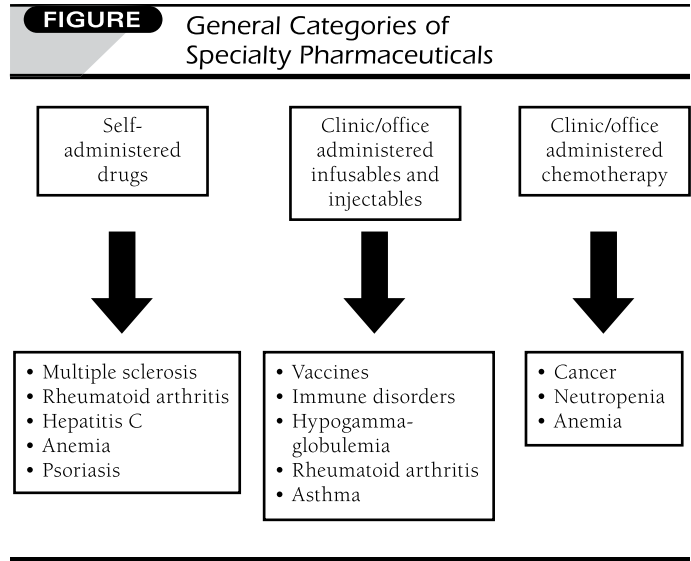
Assessing the Value of Specialty Pharmaceuticals

If targeted to patients most likely to benefit, specialty pharmaceuticals offer an attractive value proposition. These agents have the potential to decrease or reverse the progression of chronic illness and may also mitigate the adverse consequences of chronic disease. Specialty agents also have the potential to increase life expectancy, improve quality of life, enhance workplace productivity, minimize the burden of disease, reduce health care spending, and limit the overall cost of disease. If all of these benefits are true, the question becomes, are these agents worth their price?

Payers are likely willing to reimburse for specialty medications if they represent good value for the money. Payers want to know if costly specialty products are safer and more effective than drugs currently being used, and if they will prevent (or at least mitigate) higher medical costs today and in the future. Unfortunately, the ability to answer these questions is hampered by a lack of consensus treatment guidelines, robust long-term clinical data, and definitive and unbiased economic evaluations. Payers must make utilization, coverage, and reimbursement decisions with little or no information about the actual value of these agents.

Today, U.S. managed care plans and other payers are using evidence-based processes to evaluate clinical and economic data on new pharmaceuticals objectively, an approach supported by the AMCP *Format for Formulary Submissions*.⁹ These formulary submissions guidelines suggest health plans request an evidence dossier from the drug manufacturer containing detailed information, not only on the drug’s effectiveness and safety for indications approved by the FDA, but also on off-label indications and on the drug’s economic value relative to alternative therapies.

Efforts to determine the value of a specialty drug are hampered by a lack of clinical and economic data upon which to base the decision. Some of the most important clinical benefits of a drug cannot be measured in clinical trials because they are not observable for years or even decades—a period of time that far exceeds the limits of most clinical trials. Additionally, trials that analyze the clinical effectiveness of new drugs rarely contain economic information. Because calculating the financial impact



of specialty drugs is a critical step in the drug review process, payers should demand more than theoretical projections to support the proposed value of these agents. To fill this gap and to meet global payer requirements, sophisticated disease-based pharmacoeconomic models have been developed. These models do more than simply project the fiscal impact of a new product on the pharmacy budget. A well-designed model indicates the extent to which drug costs may be offset by reductions in other medical costs, evaluates the cost-effectiveness of the new treatment, and in some cases, helps identify target subpopulations where the drug will have a greater benefit and/or a smaller number needed to treat (improving incremental cost-effectiveness ratios in such patients).

Case studies provide a useful tool to describe the steps taken by payers to determine the overall value of specialty products. Two are outlined below.

The first case involves a full formulary evaluation of a newly introduced glycemic control product (exenatide). The assessment included an economic evaluation using a validated diabetes outcomes pharmacoeconomic model developed by the Center for Outcomes Research.^{10,11} The model evaluated the new therapy by determining its impact on total health care spending and created scenarios that allowed the health plan staff and the formulary committee to ascertain the clinical and economic effects of different reimbursement strategies. The model allowed for projections of the therapeutic intervention on long-term endpoints, such as myocardial infarction and hospitalizations and identified subpopulations of patients where the drug could have a greater benefit. Combined with the available clinical data provided by the manufacturer, the model outputs provided the health plan

sufficient information to support adding the drug to the formulary.¹² As evidenced from this example, the combination of model outputs and clinical data provide a robust process for assessing the value of new agents.

The second case involves the process followed by the National Institute for Health and Clinical Excellence (NICE) in the United Kingdom (UK) to determine the cost-effectiveness of using omalizumab in patients who had severe persistent allergic asthma and remained symptomatic despite treatment with a combination of high-dose inhaled corticosteroids (ICS) and long-acting beta agonists (LABA).¹³ The desired outcome was to determine the value of adding omalizumab to standard therapy. To do this, a model was developed using clinical data from 2 well-designed clinical trials (Innovate and ETOPA) and economic data from Sweden, Canada, and the Netherlands (all countries with a single-payer health care system). On the basis of the analysis, the NICE decision was that omalizumab would be recommended as add-on therapy to standard (ICS+LABA) care. More specifically, the model allowed the payer (UK NHS) to define concisely the characteristics of patients who were eligible for the drug and subsequent reimbursement. In addition, the model output provided precise criteria that described when the drug should be initiated and when it should be discontinued.¹³

Summary

Specialty drugs represent the future of pharmaceutical management of disease. These medications offer the possibility of hope for patients, providers, and payers because many specialty drugs are more effective than traditional agents. The effective management of specialty pharmaceuticals is linked closely to a distribution system designed to assure appropriate patient selection, risk mitigation, and data collection. Costs associated with these agents are projected to have a significant impact on the health care system and play a large role in determining coverage and reimbursement. Today, payers are more interested in formal and rigorous assessments of the value of these agents.

DISCLOSURES

Sean D. Sullivan discloses that there was no relationship or financial interest related to the topic of this activity. Sullivan was responsible for the entire study concept and design of this article. He performed all the data collection, data interpretation, writing, and revision of this article.

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