



## **Managing Specialty Pharmaceuticals: Balancing Access and Affordability**

*July 16, 2008  
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**Conference Report**

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## Key Themes

### Overview

The high cost and growing utilization of specialty pharmaceuticals makes them an increasingly important policy issue for patients, health plans, pharmaceutical firms, and policy makers. The key challenge for these stakeholders is finding an appropriate balance of access and affordability. At the same time stakeholders must strive to maintain a climate that encourages investment in developing new breakthrough medicines.

Payers have begun responding to the cost challenges with a range of strategies to manage spending and promote appropriate use, including benefit design, payment policy, and use of new specialty providers. A major flash point is emerging as payers have increasingly placed high coinsurance requirement on specialty drugs. How policymakers address conflicts among key stakeholders may foretell how the United States will approach future challenges of financing increasingly expensive health care for the nation's middle class.

### Context

On July 16, 2008, The Health Industry Forum brought together a group of payers, providers, and pharmaceutical firms, patient advocates, and policy experts to examine issues surrounding the future management of specialty pharmaceuticals.

### Key Themes

- **Both the clinical value and expense of specialty pharmaceuticals are poised to grow rapidly.**

Specialty pharmaceuticals including high-cost drugs and biologics currently account for about 20% of total drug spending with total costs growing by 20% annually. Other key issues related to specialty drugs include:

- *Overlapping coverage.* Some of these drugs are covered under a sponsor's pharmacy benefit; some are covered under the medical benefit. Sometimes the same drug is covered under both benefits, depending on where it is administered and who administers it. Provider reimbursement and patient coinsurance often differ in the pharmacy and medical benefit, resulting in significant complexity, inconsistent medical management, and misaligned incentives for patients, and providers.
- *High patient cost sharing.* Because these drugs are so expensive, payers frequently group them as part of a separate tier. Within this tier, coinsurance is common, and patients are often required to pay 25% to 33% of the cost of these drugs, which can be thousands of dollars per year out-of-pocket.

As the use of these drugs grow (and more such products developed and launched), the total costs of specialty drugs will further accelerate.

- **Payers are developing new approaches for managing the cost and appropriateness of specialty drugs.**

Among the strategies being used are:

- *Traditional utilization management.* This includes developing guidelines for who should receive a drug, prior authorization, expanded clinical reviews, and tiered formularies.
- *Provider reimbursement.* Some payers are looking to decrease physicians' ability to "buy and bill." These payers desire to pay physicians for their services but eliminate physicians' margins on drugs.
- *Specialty pharmacies.* This focused distribution channel provides payers with greater control, more information, and in some instances lower costs.

While some of these strategies are yielding positive results, cost trends remain high.

- **Key policy issues raised at this Forum need to be addressed.**

- *Benefit structure.* Payers and policymakers evaluate whether separate medical and pharmaceutical benefits makes sense for specialty products, since some of these drugs cost as much as a major medical procedure. Potentially the drug and medical benefit could be integrated for specialty pharmaceuticals or some hybrid benefit structure adopted.
- *Access and affordability.* Coinsurance for specialty drugs creates a heavy financial burden on patients, inequitably distributing costs onto the sick versus the well. The following policy options were mentioned: 1) continuing the current coinsurance structure (patients paying 25% or 33% of the cost) with subsidies for low-income patients; 2) requiring payers to cover these drugs for whoever needs them; 3) nationalizing this risk through a federal reinsurance program; or 4) designing more effective management programs supported by improved comparative effectiveness research.
- *Diagnostics and biomarkers.* There was much interest in the development of tests to more precisely determine exactly who will benefit from a therapy. However, smaller markets for a particular drug would likely increase the price.
- *New payment models.* New models such as value pricing, outcomes-focused performance guarantees, and various risk-sharing arrangements were discussed and received much interest.

Failure to develop policies that deal more effectively with these issues could result in a backlash that leads to strong calls for a national process for evaluating and approving specialty drugs (and other expensive medical technologies) based on both clinical and cost effectiveness criteria.

## Specialty Pharmaceuticals: Market Overview

Presenter: **Debbie Stern, RPh**, Vice President, Rxperts Managed Care Consultants, Inc.

### Overview

The development by the pharmaceutical and biotech industries of exciting new (and very expensive) drugs brings with it a host of challenges. In particular, both the cost and utilization of these specialty pharmaceuticals are dramatically escalating. In some instances these products are covered as part of a health plan's pharmacy benefit; in other instances they are part of the medical benefit—resulting in different reimbursement rates, cost sharing, and incentives for patients, physicians, manufacturers, and plans.

The high costs of specialty drugs are causing payers to employ strategies such as use of specialty pharmacies, utilization management techniques, and changes in benefit design. To balance access and cost in the future, there will likely be greater focus on obtaining outcomes data for specialty drugs and developing diagnostics to more precisely target therapies to patients who will benefit most.

### Context

Ms. Stern provided an overview of the specialty pharmaceuticals market. She defined specialty pharmaceuticals, described key trends, discussed coverage and benefit design issues, and offered her thoughts on the future of this market. Attendees then raised questions and shared their perspectives.

### Key Takeaways

- **While there is no singular definition of specialty pharmaceuticals, the distinguishing characteristic is their price.**

Ms. Stern provided the following working definition of specialty pharmaceuticals: A category of drugs resulting from advances in drug development research, technology, and design that typically treat chronic or genetic conditions. They can be biopharmaceuticals, blood-derived products, or complex molecules, and can be administered orally, through injection, or via infusion. Unlike traditional pharmaceuticals, specialty pharmaceuticals require substantial patient education, patient-specific dosing (and sometimes special handling), and monitoring for side effects.

Specialty pharmaceuticals typically cost from \$1,200 to \$40,000 per month, and thus are receiving significant attention from payers. Because many of these drugs are for chronic conditions like rheumatoid arthritis and multiple sclerosis, they might be used by patients for an entire lifetime, with serious financial implications for the health system.

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*"Really, the definition [of specialty pharmaceuticals] is about money. If these products cost \$100 per patient per month, we wouldn't be having this conversation."*

— Debbie Stern, RPh

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- **The key specialty pharmaceutical trends are increasing costs per patient and increasing utilization.**

- *Increasing cost per patient.* Because many specialty products have few competitors (and there are no generics), manufacturers are able to exert more leverage on pricing. This is seen through high launch prices and annual increases of 10% to 15% in some categories.
- *Increasing utilization.* Multiple factors are combining to increase utilization of specialty pharmaceuticals including: a flourishing pipeline of specialty drugs; research pointing to more indications for existing drugs; off-label use; earlier use of biologics in treatment; and a shift from being used to treat rare diseases and episodic conditions to treating more common, chronic diseases.

The result of these two trends is that total spending on specialty pharmaceuticals has increased dramatically. While over the past four years the cost of traditional drugs has increased at roughly 3% to 6% per year, the cost of specialty pharmaceuticals has grown 18% to 20% per year—and is forecast to continue growing at this rate.

- **Payers are increasingly using specialty pharmacies to help manage cost and promote appropriate use of these products.**

There are multiple channels through which specialty pharmaceuticals are distributed, administered, and billed. The most common ways in which specialty drugs are administered are:

- *Self-administered.* In this situation, a physician writes a prescription, which the patient has filled at a retail pharmacy or through a mail order pharmacy. The patient pays the copay and the pharmacy bills the patient's health plan. The patient then self-administers the medication.
- *Office-administered.* In this situation, a physician purchases the drugs from a manufacturer or wholesaler, and administers the medication to the patient in his or her office. Physicians bill the patient's health plan for drugs (which the physician has marked up) and for their services. For many physicians this has been a lucrative revenue stream. Similar processes exist for specialty pharmaceuticals that are administered through outpatient hospital and home infusion channels.

Specialty pharmacy providers (SPPs) represent a relatively new and increasingly prominent distribution channel. These specialized pharmacies specifically focus on the supply and management of specialty drugs. SPPs engage in:

- *Distribution.* They distribute specialty drugs to patients, physicians, and other parts of the health care system.
- *Clinical services.* They provide tailored clinical services for patients, such as compliance monitoring and proactive refill management. In addition, SPPs provide services such as

therapy management, utilization management, and formulary management.

- *Reimbursement services.* SPPs can coordinate billing, manage prior authorization, conduct benefits investigations, handle appeals, and deal with other reimbursement issues.

The maturation of SPPs is affecting how specialty drugs are managed. Some pharmaceutical companies are limiting the distribution of their specialty drugs to a few select SPPs. Most health plans are allowing patients to obtain self-administered injectables through SPPs, however, some are making SPPs the sole channel through which specialty drugs may be purchased. Also, some managed care plans are requiring that physicians order their office-administered specialty drugs through an SPP; the health plan then pays the SPP directly for the drugs and pays the physician only for administration. This change eliminates a significant revenue stream for many physicians.

- **Payers are trying to improve their ability to control specialty pharmaceutical spending through a range of medical management and benefit design strategies.**

Among the challenges payers are wrestling with is that specialty drugs are covered under different benefit structures. In some instances, specialty drugs are part of a health plan's pharmacy benefit; in other instances, they fall under the medical benefit. (This has resulted in a silo mindset; needed is a more holistic perspective.) The benefit under which a drug is covered is based on factors such as who administers the drug and where it is administered. Drugs covered under different benefit structures have different reimbursement rates, cost sharing, clinical review processes, and degrees of medical management. This can cause a misalignment of incentives among physicians, patients, health plans, and SPPs. For example, oncologists may have a financial incentive to administer an infused chemotherapeutic rather than an oral alternative because they can make more profit on the infused drug's markup.

Strategies that payers are deploying to try to control the costs of specialty drugs include:

- *Utilization management.* Payers are developing clinical guidelines and criteria for when patients should receive specific specialty drugs. They are requiring prior authorization to ensure appropriate use and to try to control off-label use, and are promoting formularies with preferred products (and different copays). The intent is to limit specialty drugs to just those patients who would benefit from them. To that end, some payers limit the quantity of the initial prescription to then re-determine medical necessity based on the patient's initial response to the drug.

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*"Payers want to ensure appropriate utilization, which mean getting the right drug in the right dose to the right patient at the right time."*

— Debbie Stern, RPh

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Ms. Stern gave an example regarding psoriasis. For every 1,000 people in the general population, 26 will be diagnosed

with psoriasis. These 26 will be treated with conventional options. Of the 26, on average, 6.5 of them will have a more severe form of the disease and should receive systemic medications. And, of this 6.5, 1.6 of the patients are at the highest risk for this disease and should receive the most advanced biologic therapies. The challenge payers face is ensuring that only the appropriate 1.6/1,000 patients receive the appropriate specialty drug.

- *Changing the benefit design.* There is a slight trend among health plans of moving specialty drugs out of the medical benefit and into the pharmacy benefit. At the same time, some payers are exploring increasing patient cost sharing by creating a 4<sup>th</sup> copay tier. This could ultimately include deductibles, out-of-pocket maximums, and annual and lifetime maximum benefits.

- **Payers will increase their efforts to measure outcomes, identify appropriate patients, and create payment models where manufacturers share risk for patient outcomes.**

The high cost of specialty drugs and the increased attention to them now will drive changes in the industry. Among the possible changes:

- *A focus on outcomes.* To date, only limited outcomes data are available when a new drug comes to market, but payers will increasingly demand "pharmaeconomic value" from new therapies. This will require data demonstrating outcomes.
- *New payment models.* It is conceivable that payment for specialty drugs could move towards increased risk sharing or pay-for-performance models where manufacturers might rebate payers if outcomes fell short.
- *An emphasis on targeting optimal therapy.* For any drug, only a fraction of those receiving it may benefit; many patients who receive a drug do not show a positive response and some experience adverse effects. To optimize the use of specialty drugs, more companion diagnostic tests will be developed to prospectively identify patients most likely to benefit.

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## Participant Discussion

- **Risk sharing.** Participants were quite interested in new financial models, but none were aware of such models currently in use in the U.S. One participant suggested thinking more broadly than just refunds (money-back guarantees) or P4P. She suggested that there are multiple forms of possible risk-sharing arrangements.

## Specialty Pharmaceutical Management Strategies: Commercial Market

Presenter: **Margaret M. Johnson, RPh**, Vice President, Chief Pharmacy Officer, Horizon BCBS of New Jersey

Respondent: **Scott Howell, MD**, Senior Director of Contracting Strategy, Genentech

### Overview

Commercial health plans are employing a variety of strategies to ensure appropriate use of specialty drugs and control costs. Among these strategies are utilization management, provider reimbursement, use of specialty pharmacies, and benefit design. Officials from the biopharmaceutical industry voiced cautious support for these strategies, particularly those focused on appropriate use. One firm, Genentech emphasized its goal of “creating targeted molecules for targeted conditions”, with companion diagnostics that help indicate which therapies will work best for which patients.

### Context

Ms. Johnson provided a commercial payer’s perspective on managing specialty pharmaceuticals. Dr. Howell responded to Ms. Johnson’s remarks and shared the views of a leading manufacturer of specialty drugs. Forum attendees then offered comments.

### Key Takeaways (Johnson)

#### ■ Managing specialty pharmaceuticals is a priority for commercial plans.

Ms. Johnson indicated that health plans look at specialty drugs within the context of managing large, high-cost patient populations. A survey of health plans indicated the highest-priority populations as those with diabetes, hyperlipidemia, asthma, cancer, hypertension, depression, substance abuse, and rheumatoid arthritis. For many of these populations, specialty drugs are an important (and expensive) part of their care. On average, specialty pharmaceuticals are used to treat 1% of a commercial health plan’s population, yet account for 11% to 13% of total drug costs. The cost trend is increasing at 12% to 15% per year.

Currently, two-thirds of health plans cover self-injectable drugs under the pharmacy benefit; the rest cover them under both the medical and pharmacy benefit. About 70% of plans cover clinician-administered injections under the medical benefit, with the balance covering them under both the medical and pharmacy benefit. About 5% of plans have a separate rider.

Health plans employ a variety of strategies to manage specialty drugs. Ms. Johnson indicated that Horizon BCBS of New Jersey’s top priorities are clinical and utilization management, provider reimbursement, use of specialty pharmacies, and benefit design.

1. *Clinical and utilization management.* At Horizon BCBSNJ, responsibility for utilization management is shared by the pharmacy and medical departments, and the approach is collaborative and holistic. The key areas of focus include clinical guidelines, prior authorization, dispensing limits, and

dose optimization. The areas of “medical necessity” and “re-determination” receive a great deal of attention as a way to help control off-label use.

Among the key challenges are lack of good data on outcomes, ROI, and costs/benefits. This illustrates a pressing need for comparative effectiveness research. Also needed are better diagnostic tools for targeting therapies; however, plans must factor the greater use of such diagnostics into benefit design.

2. *Provider reimbursement.* Horizon BCBSNJ has taken several steps on this front. This includes establishing specialist contracting guidelines; changing how physician “buy and bill” works, reimbursing based on physicians’ actual acquisition cost; and paying physicians for administering a drug, which reduces the plan’s cost by eliminating physicians’ margin on specialty drugs. Plans are also taking into account cost differentials for different therapeutic classes and sites-of-service (e.g., physician offices vs. hospital outpatient clinics) when developing reimbursement policies.

3. *Specialty provider strategies.* In New Jersey, “any willing provider” laws impact a plan’s ability to limit its pharmacy network. However, Horizon BCBSNJ has worked with the state to establish credentialing requirements for dispensing specialty pharmaceuticals. This is based on Horizon’s view that dispensing these drugs requires expertise and special capabilities in terms of safety, patient education, and reporting.

Among Horizon BCBSNJ’s specialty provider strategies has been mandating the use of specialty pharmacies by patients purchasing drugs for self-injection, and making an exclusive specialty pharmacy option available to employers. Horizon has also contracted with oncology-focused specialty pharmacies to provide same-day service to physician practices. In addition, Horizon is, in some instances, contracting with drug manufacturers both directly and through intermediaries. These contracts help make pricing more competitive and also include provisions dealing with outcomes.

In working with specialty pharmacies, health plans expect: programs based on clear guidelines; consistent application of such programs; adherence to formulary guidelines; management of waste and inventory; patient-centric efforts that focus on educating patients, optimizing the therapy provided, and ensuring adherence; serving as a resource that extends the plan’s complex case management team; and plan-specific reporting.

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*“We expect specialty pharmacies to be part of our holistic approach [to patient care].”*

— Margaret M. Johnson, RPh

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4. *Benefit design.* For Horizon BCBSNJ, benefit design means more than just cost sharing. The goal is to design benefits that meet the varying demands of employers and patients. For specialty products, Horizon's experience indicates that copay differentials may not be sufficient to drive use of preferred products (a \$20 or \$50 copay may not matter for a drug that is over \$1,000 per month). However, because New Jersey regulations prohibit a higher copay tier for specialty drugs, this is not an option for Horizon BCBSNJ. One other important area in benefit design is vaccines. Historically vaccines have been preventive and relatively inexpensive. As the vaccine market evolves, plans may have to modify their benefits.

## Key Takeaways (Howell)

In providing a biotech perspective, Dr. Howell structured his remarks on the four areas that Ms. Johnson addressed:

1. *Clinical and utilization management.* Genentech strongly supports efforts such as utilization management that promote appropriate use of specialty therapies. Genentech seeks to promote appropriate use by:
  - Developing improved labels for its drugs.
  - Engaging in "on-label" promotion.
  - Requiring that all clinical development programs have a companion diagnostic strategy to identify which subset of patients will benefit from the therapy.
2. *Provider reimbursement.* As a manufacturer, Genentech generally stays out of provider reimbursement issues. The only comment Dr. Howell offered in this area is that financial incentives should not distort delivering the right care.
3. *Specialty provider strategies.* Genentech has observed significant changes in the specialty pharmacy sector. Originally specialty pharmacies were focused on services such as patient education and administration. As the industry has consolidated, these providers seem increasingly focused on "customer management," becoming more of an agent for the insurance plan rather than an advocate for the beneficiary.
4. *Benefit design.* Genentech supports benefit design structures that provide patients with unfettered access to the right treatments. The company is concerned with trends toward higher deductibles and copayments, which could hurt the ability of some patients to access the treatments they need. Genentech has programs that augment access, including copayment assistance programs and programs that offer free prescriptions.

## Participant Discussion

- **Potential backlash.** Stuart Altman expressed concern that what is happening with specialty pharmaceuticals mirrors what took place with managed care in the 1990s. High medical costs caused managed care to tightly manage utilization while increasing consumer out-of-pocket costs. The result was ultimately a significant backlash against managed care.
- **Denying access.** A representative from the pharmaceutical industry suggested that once a potentially life-saving therapy is developed, it is untenable for payers or society to limit access to this treatment.
- **Social solidarity.** Is there a point at which insurers should refuse to cover a proven beneficial therapy because the benefit gained is not worth the cost? Countries such as Germany engage in "social solidarity" where a central entity makes a decision regarding whether a drug will be covered or not, and how much the government will pay. If a treatment costs too much or provides too little benefit, the treatment is simply not covered. In this way, the country's tries to use its resources in an efficient way. However, this could result in potentially life-saving therapies not being available to some patients because the cost is too high.

The question was raised whether this model could ever exist in the United States. Professor Uwe Reinhardt suggested that the US government might simply impose a QALY threshold; any treatment where the cost was over that threshold would be deemed too expensive and wouldn't be paid for.

- **Incenting innovation.** Participants commented that imposing such cost effectiveness limits on reimbursement or coverage would hinder innovation because it would decrease incentives for investment by pharmaceutical and biotech companies. Also, many of medicine's most important innovations result from a series of small improvements on products that may have initially demonstrated marginal performance. Policymakers need to be cautious that policies and changes to reimbursement don't eliminate the incentives needed for innovation.
- **Underpayment.** While purchasers are frequently concerned about overutilization of specialty drugs, there are also many instances of underutilization, often through under-dosing. When appropriate guidelines are put in place, utilization of certain specialty therapies may actually increase.
- **Adverse selection.** Health plans that continue offering a three-tier benefit design in an environment where other plans are offering four- or five-tier programs will face adverse selection. By offering less-restrictive formularies, the health plan will disproportionately attract patients that need these expensive specialty products, resulting in financial losses or uncompetitive premiums levels.

## Specialty Pharmaceutical Management Strategies: Medicare & Medicaid

Presenter: **Dan Mendelson**, President, Avalere Health

Respondent: **Matt Eyles**, Vice President for Public Policy, Wyeth

### Overview

Government payers face similar challenges in managing specialty pharmaceuticals as commercial plans, and drugs, and are adopting many of the same strategies. Key challenges include the high costs of specialty pharmaceuticals, the potential patient burden of co-insurance, and the confusing and conflicting incentives created by overlap in medical benefits and pharmaceutical benefits.

Medicare Part D is administered by private plans and therefore utilizes private sector strategies within the context of the program regulations. These strategies include prior authorization, utilization management, tiered formularies, and specialty pharmacies. CMS has less flexibility under Medicare Part B and uses a much more limited tool kit (e.g., such as coding decisions) to manage spending. Significant change in how CMS manages specialty drugs would require changes in both regulation and statute. Several policy options ideas were presented including proposals to reduce patient financial barriers and greater use of comparative effectiveness research.

### Context

Dan Mendelson provided an overview of how specialty pharmaceuticals are managed in Medicare and Medicaid, and identified key challenges. Matt Eyles shared Wyeth's perspective on specialty pharmaceutical challenges, and discussed potential public policy solutions.

### Key Takeaways (Mendelson)

- **Medicare's management of specialty drugs faces similar structure and challenges to those of commercial plans.**

Medicare Part B is analogous to the commercial plans' medical benefit and includes coverage for drugs administered in a physician's office or another medical facility, while Part D is similar to commercial plans' pharmacy benefit, and is for self-administered drugs. Additionally, as with commercial plans, some drugs are covered under both Part B and Part D, with different rates of reimbursement, and differing incentives for providers.

*"Depending on clinical circumstance or site of service, some drugs can be covered both under Parts B and D."*

— Dan Mendelson

There are few clinical guidelines regarding which drugs should be used and how or where they should be administered, leading to much confusion.

- **CMS uses some of the same tools and strategies as commercial plans to control costs, though its flexibility is limited under Medicare Part B.**

Medicare has a more limited set of tools for managing specialty pharmaceuticals under Part B than do private payers. Utilization controls are focused on specific coverage and payment determinations, since formularies are not used in Part B. Other tools include:

- *HCPSC coding decisions.* Medicare can decide whether to create a unique code for a product or to have multiple products share the same code. Grouping multiple products together under the same code allows the agency to blend the prices and create one reimbursement rate. To date, CMS has not used the technique very aggressively.
- *Least Costly Alternative (LCA).* Even with separate codes for specific products, Medicare can limit reimbursement to the least costly alternative product. This policy is a threat that Medicare can use, but Medicare hardly ever chooses to do so. There is congressional opposition to greater usage.
- *Formulation coverage restrictions.* Contractors can deny Part B coverage if a self-administrable formulation is available under Part D.
- *Competitive Acquisition Program (CAP).* The intent of this program is to get physicians out of the business of buying, billing, and making profit from drugs. However, CAP is currently an optional program (only about 4,000 Medicare providers have enrolled) and does not create a formulary. As a result it has a limited ability to control utilization.

Medicare Part D is administered by private health plans that utilize a wide array of approaches:

- *Utilization management.* These tools include prior authorization, quarterly limits, and step therapy. In general, Part D plans use more intensive utilization management than typical commercial programs.
- *Tiering.* Medicare Part D plans have implemented more aggressive tiering structures than commercial plans, with more than 85% of plans having four or five tiers. The fourth and fifth tiers typically have cost sharing for specialty drugs where patients must pay 25% or 33% of the cost.

Some patients attempt to save money through a practice termed "brown bagging." Patients purchase a product directly through a retail channel and take it to a physician's office to be administered. While this may decrease patients' out of pocket costs, it raises safety issues (if, for example, patients transporting the drugs leave them in a hot car).

▪ **Many states are looking into new specialty drug management programs to control Medicaid spending.**

Specialty drugs are also on the radar of state Medicaid agencies but there is wide variation in actual practice. Currently, most states do not have relationships with specialty pharmacies, but some are beginning pilots and other states are looking into such programs.

▪ **Public payers must wrestle with many vexing policy issues concerning, cost, access, and financial incentives.**

Among the policy debates that need to occur are debates around:

- *Cost.* Will society accept public benefits programs that require persons with serious illnesses to pay 33% of the cost of a specialty drug, which could conceivably be thousands or tens of thousands of dollars per annually?
- *Access.* Can utilization controls (such as step therapy and pre-authorization) reduce inappropriate use of specialty pharmaceuticals without impeding the ability of patients who are truly needy to acquire them?
- *Pricing.* Can programs develop new payment models like capitation for specialty drugs where suppliers provide all of the drugs a payer needs for its enrollees for a fixed annual fee.
- *Benefit design.* Should payers create a level playing field with consistent coinsurance and payment rates for the same drug under both a medical and pharmaceutical benefit?
- *Physician incentives.* The current system provides various economic incentives to physicians. (For example, a physician might make a profit of \$2,000 for prescribing and injecting one drug over another.) Should public policy ensure that all parties, including physicians, have the incentives to use the appropriate drug for the appropriate patient?

## Key Takeaways (Eyles)

Matt Eyles shared Wyeth's perspective on some of the challenges surrounding specialty drugs and some solutions to these challenges.

▪ **Wyeth sees several important issues related to specialty drugs, but believes there are solutions.**

Wyeth has long been concerned with issues of patient access to specialty drugs, as well as affordability and out-of-pocket spending. Wyeth does not advocate coverage without limits but rather rational tools for managing utilization. Mr. Eyles shared data indicating that 59% of specialty drugs in Medicare Part D are subject to prior authorization versus just 12% of traditional drugs—statistics which show that utilization management is prevalent.

In the current Part D program, Wyeth sees the following issues:

- *Barriers to access.* At the moment, cost serves as the primary barrier to access for specialty drugs.

- *Restrictions on appeals and exceptions.* There are important limits on a patient's ability to make timely appeals for exemptions to specialty drug cost sharing.
- *Lack of standard language.* There is no standard terminology for specialty drugs, resulting in beneficiary confusion.
- *Decreased benefit value.* The cumulative impact of these issues—barriers to access, restrictions on appeals, and confusing language—is that some beneficiaries may be at a disadvantage, decreasing the overall value of the Part D benefit.

Wyeth does see potential solutions for these issues. In addition to revising the appeals/exceptions process and standardizing benefits language, Mr. Eyles suggested several options for decrease barriers to access. One would be independent review of appropriateness that would indicate when a drug is highly appropriate. Another would be allowing greater flexibility in beneficiary cost sharing such as: a "lesser of" policy which allows patients to pay the lesser of a copayment or coinsurance; or an arrangement that would allow beneficiaries to have level cost-sharing for specialty drugs throughout the year (rather than a large lump sum in the donut hole), analogous to heating or cooling payment programs offered by utility companies.

## Participant Discussion

- **Dose of perspective.** Debbie Stern reminded participants that just a few short years ago Part D didn't even exist. Yes, issues remain, but tremendous progress has been made, and the issues now facing Medicare/Medicaid are the same as those facing commercial plans.
- **Need for comparative effectiveness data.** Several participants agreed that comparative effectiveness data is needed. This data will provide credible information to help guide decisions on which specialty drugs should be used, for which patients.
- **Can we say "no?"** Stuart Altman pointed to a key issue in this debate -- whether the United States will ever be willing to adopt the practices of other countries that refuse to cover drugs that lack incremental value compared to existing treatment options.

## Social Policy Issues for Controlling Specialty Pharmaceutical Spending

Moderator: **Stuart Altman, PhD**, The Heller School, Brandeis University

Panelists: **Kathy Buto**, Vice President for Policy, Johnson & Johnson  
**Edmund Pezalla, MD**, National Medical Director and Chief Clinical Officer, Aetna Pharmacy Management  
**Joseph Jacobson, MD**, Chairman of Medicine, North Shore Medical Center  
**Bari Talente**, Associate Vice President, State and Local Government Relations, National Multiple Sclerosis Society

### Overview

Stakeholders have widely different views of potential approaches to managing specialty pharmaceuticals. Patient advocates are concerned with access and cost; physicians have come to rely on revenues from re-selling specialty drugs, and argue that although the cost of these drugs is significant, so are the benefits; payers are increasingly using tiered formularies and utilization management to control spending, but believe that society needs to decide how to collectively cover these costs; and pharmaceutical manufacturers want to maintain incentives for innovation. All stakeholders agree that society must more explicitly take on questions of balancing access, affordability, and innovation. Participants discussed a variety of approaches (such as value-based pricing and coverage with evidence development) that need further development.

### Context

Each panelist offered thoughts about key issues for managing specialty pharmaceuticals, and offered policy options. Participants then shared their own perspectives.

### Key Takeaways (Buto)

Kathy Buto provided a perspective from a pharmaceutical manufacturer.

- **The dialogue around specialty pharmaceuticals needs to be broader than just "controlling costs."**

The primary focus of conversations about specialty drugs is, "How can we control the costs?" But this focuses the conversation too narrowly. A more appropriate framing would be on balancing access and affordability by focusing on value and appropriate use.

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*"It seems that people often just talk about spending and just talk about drugs."*

— Kathy Buto

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- **Regulatory requirements and business model requirements affect the high cost of specialty drugs.**

It is important that policymakers understand why the costs of drugs are what they are, and what factors affect drug prices:

- *Regulatory requirements.* Due to concerns about safety and toxicity that are often seen once a drug has been in the market for some period of time, regulators are demanding more research prior to approving a drug. This lengthens the development cycle and increases development costs. (This regula-

tory direction runs counter to pharmaceutical R&D strategies that are trying to shorten development cycles.)

- *Pricing realities.* Manufacturers frequently set their initial "launch price" on the high side, perceiving that they will never be able to increase their price without a payer or patient backlash.

- *Business model issues.* While use of biomarkers and diagnostics will enable better targeting of therapies, they will also decrease the size of the market for some therapies. Smaller markets require higher prices to cover the development costs.

The traditional pharmaceutical business model has been marketing to physicians to encourage prescribing to (hopefully) large numbers of patients. But payers increasingly want to pay only when a drug works. This will significantly affect how the pharmaceutical industry develops drugs and sets prices.

- *International markets.* Global market dynamics increasing affect the US market. For example, expansion of reference pricing in Europe creates increased pressure to raise US prices. Some companies may have policies to virtually give away drugs for some diseases or geographies (for example, AIDS drugs for South Africa), contingent on achieving certain price levels in the US. International markets may also affect the pharmaceutical industry based on the science coming from countries like China and India. It is not out of the realm of possibility that as the science improves in these countries, the US pharmaceutical industry could be diminished.

- **New payment models can help balance the issues of cost, access, affordability, and innovation.**

Possible options include:

- *Performance agreements.* This encompasses various types of risk-sharing arrangements and outcomes guarantees.

- *Conditional approval.* There may be ways to share risk by bringing new products to market with conditional approval or coverage with evidence development.

- *Capitated payments.* Payers could negotiate capitated agreements with pharmaceutical manufacturers.

- *Patient education.* Equipping patients with data on costs and outcomes so that they can make more informed decisions is an important step in balancing access and affordability.

### Key Takeaways (Pezalla)

Dr. Pezalla offered a payer perspective on managing specialty drugs.

▪ **Tiered formularies don't work for specialty drugs.**

The three-tier formulary has been tremendously effective, for common drugs and diseases. Generics provide a low-end option and patient's have choice about what drug to purchase at what price. The three-tier model impacts patient behavior and helps control costs. But the model breaks down for specialty pharmaceuticals. Based on their cost, specialty drugs immediately go into the fourth tier. Generics don't exist, patients have no low cost choices, and the tiered concept has little effect on patient behavior. A new and different approach is needed.

▪ **Society needs to decide how to cover the costs of specialty pharmaceuticals.**

As a payer, Aetna realistically can't deny coverage for a patient who needs an expensive specialty drug. But as more patients use more expensive drugs, it will increase the cost of health insurance. It also puts patients who need such drugs in the difficult position of having to pay a significant amount. This raises equity issues and will force society to grapple with how these products will be paid for in the future. Will they be spread across all citizens or nationalized in some way? Society must confront these issues.

## Key Takeaways (Jacobson)

Dr. Jacobson, who was a practicing oncologist for 25 years, gave the perspective of a physician who administers specialty drugs.

▪ **While the costs of many drugs have increased substantially, so too have the benefits.**

Analysis has shown that the cost of treating many types of cancer patients has increased significantly; the key factor being a large increase in the cost of drugs. However, these cost increases have been accompanied by measurable and significant improvement in survival. For many types of cancer, survival that was previously measured in weeks is now measured in months and years. This increased survival is directly related to new drugs.

▪ **While oncologists generate revenue from administering specialty drugs, they often reinvest it in patient care.**

Among private oncology practices, about 70% of revenue typically comes from administering drugs. However, practices typically use these revenues to cover critical services that are not reimbursed such as hiring social workers and creating patient assistance processes that work with patients in need to help get free or lower-priced care. Current oncology practices recognize that they cannot continue to operate without the income from drug administration.

▪ **Many oncology practices are participating in voluntary quality improvement programs.**

The American Society of Clinical Oncology (ASCO) has undertaken an effort to improve the quality, and as a by-product, the cost-effectiveness, of oncology clinics. The Quality Oncology Practice Initiative (QOPI) is an oncologist-led, practice-based,

quality improvement program where 375 oncology practices are voluntarily working to gather data that will identify opportunities for quality improvement. An expected outcome from QOPI is the ability to identify areas of over- and underutilization. For example, data indicates that about 20% of patients who receive chemotherapy get it in the last two weeks of their life, which in many instances represents overutilization.

## Key Takeaways (Talente)

Ms. Talente presented a patient perspective, describing issues faced by a person with multiple sclerosis.

▪ **For patients with chronic diseases, the financial burdens of specialty drugs can be extremely high.**

The 400,000 people in the US who have multiple sclerosis (MS) tend to be diagnosed when young (20 to 40). They are typically female, educated, married, and with children. And, while most (95%) have health insurance, 70% still have difficulty paying for their health costs.

This is because the most common therapies for MS are expensive, costing roughly \$2,500 per month. With 25% to 33% coinsurance, the cost to patients is often in the range of \$500 to \$800 per month for just one drug. Because MS patients are often taking many drugs, their monthly out-of-pocket drug costs are often \$1,000 to \$1,500.

As of result, it is not uncommon for patients to have difficulty with copayments and to stop taking the drugs. In many instances this increases total costs to society because going off of therapy can lead to increased hospitalization, result in lost work time, and increased disability claims.

## Participant Discussion

▪ **Lifetime limits.** A key issues is that patients on specialty drugs can use up their lifetime insurance limits. Several perspectives were discussed.

— *Patient.* A patient who has expended their lifetime limit is essentially uninsured, creating a dire situation for a sick patient. As the cost of health care grows, this situation will become more common.

— *Insurer.* Such limits are necessary to quantify risks and set prices. With no limits, the risks would be greater and premiums would be higher.

— *Drug manufacturer.* If a patient loses coverage, many pharmaceutical companies provide patient assistance and free or reduced-price drugs. However, this doesn't address expenses associated with physician administration and other health care costs.

— *Policymakers.* We should consider policies that increase the lifetime limits and consider making the federal gov-

ernment the reinsurer of this risk. (This is comparable to what has occurred with dialysis).

- **Value-based pricing.** In Stuart Altman's view, there are three possible ways to deal with specialty drugs: 1) provide unlimited access and coverage regardless of cost; 2) continue with patient coinsurance, which provides some relief to payers but often has negative implications for patients; and 3) pursue value-based pricing, where the decision whether to pay for a treatment is based on value, not just cost. This requires gathering the necessary data to assess value.
- **Off-label use.** Rampant off-label use is a reality, and managing it is a major issue. Essentially, physicians are

deviating from the standard of care to write prescriptions—based on their experience and judgment—but without data. While off-label use has led to innovation, it can also result in harm. What is needed is the ability to collect data on off-label use. The solution may be a "coverage with evidence" approach.

- **Shared decision making.** Today the health care system does a poor job of informing patients of the costs, benefits, risks, and potential adverse consequences of a treatment. Data has shown that in general, when patients are fully informed, they usually prefer a more conservative, less costly course of action. Increasing the use of shared decision making should be a societal priority.

## At What Price Better Health?

Speaker: **Uwe Reinhardt, PhD**, Professor, Princeton University

### Overview

The debate over the cost of specialty pharmaceuticals is just one part of a larger policy debate regarding the future financing of health care for the poor and middle class. This discussion must tackle questions such as whether the more affluent members of society will pay for health care for the less affluent, whether society should impose limits on what it is willing to spend, and whether these limits should be uniform or should vary for different members of society. The discussion must take place on the demand side of health care among those who purchase products and services. When the demand side comes to a point of view and signals the supply side regarding what it wants and is willing to pay, the supply side will respond accordingly.

### Context

Professor Reinhardt shared his thoughts on the specific issues related to specialty drugs, and commented on the problems facing the U.S. health care system in general.

### Key Takeaways

- **The problem the country faces is not just how to pay for specialty drugs; it is how to cover health care for the poor and middle class.**

Professor Reinhardt is not among those who are deeply concerned that health care cost trends are unsustainable. Over the past 40 years, health care spending has grown 2.5% faster per year than the rate of GDP growth. If this were to continue through 2050, health care would represent 40% of the US economy. While alarming to many, if that level of spending meant the elimination of diseases like cancer or Alzheimer's, it might be a deal that society would take. (Even with 40% of the economy devoted to health care, the other 60% would still be quite large.)

In Professor Reinhardt's view, a more pressing problem is the unequal distribution of income and wealth in this country. Some telling statistics:

- The top 20% of earners generate 60% of the country's income and the bottom 40% account for just 10%.
- Over the past 20 years, almost all GDP growth has gone to the top 10% of U.S. society.
- The top 20% of society holds 85% of the country's wealth and the bottom 40% has virtually no wealth at all.

Essentially there are four classes in the US:

- *Aristocracy*. They reside at the very, very top and live in a separate economic world.

- *Professionals*. These are households where the husband and wife work and earn a combined income of around \$300,000.
- *Middle class*. The household might earn \$50,000, with husband and wife both working at places like The Home Depot.
- *Poor*. These individuals have incomes and lives similar to those in third-world countries.

Historically the U.S. government has done much for the poor, through Medicaid, food stamps, and housing subsidies. However, little has been done for the middle class. In the next decade, the country must decide whether or not to help this segment in paying for health care.

Some data to illustrate the situation: Today, health benefits cost \$15,600 for a middle class family of four earning \$50,000, meaning that health benefits are 31% of wages. Over the next ten years, health benefits will grow faster than income and will represent 51% of wages for this family. Thus, the challenge faced by our society is not just how to deal with specialty drugs, but how to provide health benefits to the middle class.

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*"In the next decade we have to either write the middle class out of health care or tax everyone to pay for it . . . Will the top one-third pay more taxes to help the other two-thirds?"*

— Uwe Reinhardt

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- **Health care purchasers need to think and act differently.**

Suppliers of health care products and services have acted rationally, offering products and programs that the market demanded and was willing to pay for. If change is going to occur in health care, it must be driven by the demand side. Some ideas to consider:

- *Signals are necessary*. The demand side must signal to the supply side what products and services it wants, and what it is willing to pay. The supply side will then respond accordingly. An analogy is how the Department of Defense signals its needs to defense suppliers.
- *Maximum prices*. Should the US determine a maximum price it is willing to pay for a drug or a health service? For example, in the UK, the National Institute for Health and Clinical Excellence (NICE) will usually not cover a new treatment if the cost per QALY (quality adjusted life year) is greater than £30,000. Using the Department of Defense (DoD) analogy, even if the DoD signals its interest in a particular type of jet fighter, if the cost of this fighter is \$1 billion then the DoD won't buy it.
- *Differing maximum prices*. Should the maximum price, in terms of cost per QALY, be the same for all members of society? It is common that doctors are paid one rate to see a privately insured patient and a lower rate to see a Medicaid patient. This clearly signals to the doctor that these patients

have different value. If society is going to establish different maximum prices at which it is willing to cover treatments, should those maximums vary?

▪ **As therapies become more targeted, the price per therapy will increase.**

Historically drugs have been prescribed to large groups of patients. Usually only a fraction of those receiving a drug are helped by it; many receiving a drug are not affected at all and some patients experience adverse reactions. This model worked well for pharmaceutical companies as the patients receiving a drug that didn't benefit helped finance the drug (or future drugs). However, as therapies become targeted, they will be administered more precisely, only to patients predicted to benefit. This means that pharmaceutical companies will have to charge higher prices to recoup their R&D investments across a smaller number of patients.