THE HEALTH INDUSTRY Forum

# Specialty Pharmaceuticals: Policies for Encouraging Access and Affordability

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## Conference Report

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***The Health Industry Forum*** *is based at Brandeis University, chaired by Professor Stuart Altman, and directed by Robert Mechanic. The Forum brings together public policy experts and senior executives from leading healthcare organizations to address challenging health policy issues. The Forum conducts independent, objective policy analysis, and provides neutral venues where stakeholders work together to develop practical, actionable strategies to improve the quality and value of the US healthcare system.*

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# Key Themes

## Overview

The rapid growth of specialty pharmaceuticals has raised concerns about their high costs, comparative value, and financial barriers to access, leading decision makers to seek new policy solutions. Specialty pharmaceuticals are provided through an extremely complex financing and distribution system. Policy solutions will need to consider a range of issues including: benefits structure, and the interaction between medical and pharmacy benefits, physician reimbursement, drug pricing, evidence development, and beneficiary coinsurance. The ultimately goal is to promote appropriate utilization of specialty products so that patients have access to necessary therapies in a context of coordinated patient management that also promotes high quality affordable care.

## Context

On October 2, 2008, the Health Industry Forum brought together representatives from health plans, biopharmaceutical firms, provider organizations, patient advocacy groups, and public officials to examine policy options for ensuring appropriate access to specialty pharmaceuticals while also promoting affordability. The meeting was organized to address four key challenges for specialty pharmaceuticals (as identified in the Forum's July 2008 meeting): 1) overlapping benefit structures that hinder effective medical management; 2) physician financial incentives that may bias therapeutic choice; 3) evidence gaps that hinder rational coverage and payment policy; and 4) untenable patient financial burdens created by Tier 4 coinsurance.

## Key Themes

- **Health plans and Medicare must address inconsistencies between the medical and pharmacy benefits.**

  Separate medical and pharmacy benefit structure creates confusion, and hinders effective oversight and management of specialty pharmaceuticals. As an example, current benefit structure affects how treatments are selected and administered. Beneficiaries with high drug utilization may face inequitable cost sharing under the current doughnut hole benefit structure. This issue could be addressed by creating a distinct specialty drug benefit, "Part E," or seeking other ways of bringing about greater consistency between Parts B and D that might be possible without legislation.

- **Eliminating buy and bill reimbursement could reduce problematic physician financial incentives but implementation would be extremely challenging.**

  Under "buy and bill" reimbursement, physicians can generate significant margins from mark-ups on specialty pharmaceuticals. Although there is little systematic evidence that these incentives have caused physicians to make decisions against patients' clinical interest, many believe it is more appropriate to utilize payment methods where physician compensation does not vary based on prescribing decisions. Among the options discussed was the idea of replacing "buy and bill" with an enhanced series of fees for administration and patient management. Alternatively, physicians could receive an episode fee for managing patients through specified courses of treatment. Developing these new payment structures would be technically complex, and there were concerns that declining margins could shift patients out of physician offices and into more expensive hospital settings.

- **Drug payment policy must promote development of credible effectiveness data and reward innovation.**

  While specialty pharmaceuticals can provide miraculous benefits, the effectiveness of many drugs is uncertain and may be highly variable across individual patients. There is currently no consensus mechanism for assessing the value of specialty drugs. Given the high prices for these products, there is growing demand for additional information on clinical and cost effectiveness prior to coverage. One option that was discussed was setting a product's release price based on comparable products and with mechanisms for collecting more data and adjusting prices as more evidence becomes available. Manufacturers voiced interest in a longer period of patent protection to balance lost revenues from lower launch prices.

- **Insurance must provide enrollees with adequate protection against catastrophic expenses.**

  Specialty pharmaceuticals are often defined by their high price. Most are essential, non-discretionary medications employed for chronic conditions. High prices combined with high co-payments put significant financial burdens on patients, forcing many to go without. The recent expansion of drugs covered under Tier 4 with 25 – 33% coinsurance passes the risk back to the beneficiary with potentially devastating financial and health consequences. Options for addressing this issue include establishing local laws that prohibit the Tier 4 category or establishing a reinsurance option for high cost beneficiaries that would spread the risk for these expensive products.

  All policy options must address access concerns created by the overwhelming financial burden placed on patients, including the very high cost of specialty pharmaceuticals. Policies addressing the cost of specialty pharmaceuticals should consider a pricing model that includes developing evidence of the drug's value.

# Coordinating Medical and Pharmacy Benefits for More Effective Program Management

Presenters: **Dan Mendelson**, President, *Avalere Health;* **Lauren Barnes**, Vice President, *Avalere Health*
Respondent: **Joan Sokolovsky**, Principal Policy Analyst, *MedPAC*

## Overview

Medicare's current system of managing and reimbursing for specialty drugs is the result of statutory evolution. Specialty drugs are covered under both Medicare's medical benefit (Part B) and its pharmaceutical benefit (Part D) with some drugs covered by both programs. Fragmentation of the specialty pharmaceutical benefit creates inconsistent incentives as physician payments and beneficiary coinsurance can vary significantly based on the site of service. Future policy changes should be based on guiding principles like: ensuring that patients have appropriate access to needed drugs; neutralizing physician financial incentives for prescribing one drug over another; implementing consistent utilization management controls; constant patient cost sharing requirements; and paying providers fairly for services associated with specialty drugs. A consolidated Medicare specialty drug benefit is one option for consideration.

## Context

Dan Mendelson reviewed Medicare specialty drug benefit program and identified potential concerns with the current design. He also laid out principles to be considered in designing policy solutions. Lauren Barnes presented a "straw man" proposal for modifying Medicare's specialty pharmaceutical benefit to initiate the conversation. Joan Sokolovsky presented MedPAC's perspective, and participants discussed other key considerations.

## Key Takeaways

- **Medicare's separate medical and pharmacy benefit structure challenges effective management of specialty pharmaceuticals.**

  Medicare's system for covering specialty drugs has come about through statutory evolution, and varies significantly based on site of service. There are separate medical (Part B) and pharmacy (Part D) benefits; however, substantial overlap occurs, as drugs are often covered under both Part B and Part D, with different reimbursement rates and co-pays. Specific issues include:

  - *Provider incentives.* Providers receive different payments based on whether a drug is reimbursed under the medical or pharmacy benefit.
  - *Beneficiary incentives*. Beneficiaries pay different amounts out of pocket based on whether a drug is in Part B or D.
  - *Utilization management.* There are currently tight utilization controls for Medicare's pharmacy benefit, but little to no utilization management for medical benefits.
  - *Off-label use*. There are controls on off-label use in the medical benefit but few controls in the pharmacy benefit.
  - *Brown bagging*. This is a growing trend where individuals purchase medications (such as injectable drugs) at a retail pharmacy because it is less expensive under Part D, and then takes it to a physician to be injected. This practice raises significant safety concerns (i.e., if patients leave refrigerated medications in the back of a car).

  CMS has some regulatory flexibility to change some aspects of the system, for example by defining explicitly which drugs are covered under Part B and Part D. Other changes, such as creating consistent co-pays across the benefit, would require Congressional action.

- **It is important to develop consensus around key principals before exploring policy alternatives.**

  Mr. Mendelson recommended that an important first step is identifying principles on which new policies should be based. He suggested the following principles as a starting point for a discussion with patient groups and commercial stakeholders:

  - *Neutralize prescribing incentives.* Providers should make prescribing decisions based on the merits of the therapy rather than facing financial incentives to prescribe one medication over another.

    > *"Patients want physician neutrality."*
    > — Dan Mendelson

  - *Compensate physicians/staff for medical management.* We should pay providers for what they do. If one drug requires more time and administrative effort than another, providers need to be paid more for administering the drug.
  - *Maintain equivalent patient cost sharing between benefits.* Coverage under Medicare parts B and D ought to have equivalent cost sharing.
  - *Provide immediate/timely access to medication in the appropriate setting.* A key principle of any benefit design must be timely access to drugs in an appropriate setting.
  - *Ensure consistent use of utilization controls between benefits.* There should be the same policies and practices for utilization controls regardless of whether a drug is part of Part B or D.
  - *Encourage innovative formulations/drug delivery systems.* Any change should recognize the importance of innovation.
  - *Match drug treatment to optimal setting of care.* The setting of care shouldn't be driven by financial incentives but based on optimal treatment for the patient.
  - *Ensure reasonable cost containment.* Because fiscal discipline is a CMS priority, cost containment provisions are essential. Value-based benefit design could be useful.

The key theme among these principles is the need for consistency in financial incentives, in utilization controls; and in policies and practices.

- **One option: create a consolidated specialty benefit.**

For purposes of discussion (not an Avalere recommendation), Lauren Barnes presented a consolidated specialty drug benefit option. The crux of the idea is to create consistency between Part B and Part D in regard to managing and paying for specialty drugs. Under a consolidated benefit, differential payments based on site of service would be eliminated. Providers would be paid for services related to specialty pharmaceuticals, including drug administration; medical management, counseling and treatment planning; pharmacy dispensing; and overhead fees. Utilization controls would be the same regardless of the site/setting in which drugs were delivered. And a consolidated benefit would create consistent patient co-insurance regardless of the setting where drugs are delivered.

> *"Today there are differences in Part B and Part D. The idea would be to make it consistent."*
> — Lauren Barnes

In evaluating this idea, there are several factors to keep in mind:

- *Which drugs? Policymakers would need to decide* which drugs to include in the specialty pharmacy benefit. Options include all specialty drugs or just those where there is currently overlap between Parts B and D.
- *Winners/losers.* It is important to think through who the winners and losers will be and get input from all stakeholders.
- *Scope of changes*. Targeted changes are more likely to happen quickly; broad changes in policy and structure are likely to take much longer.

While a consolidated benefit represents a big change for Medicare, participants noted that separate specialty benefits are now being implemented by commercial plans.

- **These issues are squarely on MedPAC's radar.**

Dr. Sokolovsky commented that even before Part D was enacted, MedPAC had been looking at how drugs are paid for under Part B. It is obvious that Part B creates unusual circumstances for coverage and payment. One of these circumstances is brown bagging, which occurs because physicians see this as solving a problem when they can't purchase the drug at a price equal to the Medicare payment rate.

Dr. Sokolovsky indicated that MedPAC is interested in many of the principles mentioned by Mr. Mendelson. These include site of service payment neutrality, beneficiary cost sharing, and cost containment. To date, MedPAC's recommendations have been on a case-by-case basis. For example, MedPAC has recommended that some preventive vaccines be shifted to Part B. In addition, CMS has been working to standardize use of Compendia for approval of off-label use of drugs under both the medical and pharmacy benefit.

## Participant Discussion

- **Some specialty drugs aren't discretionary.** Dr. Sharon Levine pointed out that specialty drugs as a category are not discretionary. Because of this, having patients share the costs of these very expensive drugs as a strategy to control utilization is not appropriate.
- **"Part E."** A clean way to think about a new and distinct specialty drug benefit is creating a "Part E." However, establishing a "Part E" would require legislation, while other ways of bringing about greater consistency between Part B and Part D might be possible without legislation.
- **Bundling.** Specialty drugs are often delivered as a "package" involving both the drug and the delivery of services (i.e. cancer drugs). Providers like oncologist often receive very limited payment for delivering services; instead they earn income from drug markups. Policymakers need to think about whether payment for specialty drugs can and should be separated from services, or whether there could be a bundled payment like DRGs.
- **Paying for cognitive services.** While the discussion focused mostly on payments to oncologists for services, participants noted that internists and other physicians also aren't adequately compensated for cognitive services. The issue is broader than specialty drugs; it is fairly paying all providers for services.
- **ESRD analogy.** Dr. Jack Rowe suggested looking at ESRD as a potential model for specialty drugs. ESRD does not provide for a bundled payment; drugs (i.e. EPOGEN) are paid for separately from services, and nephrologists receive a per patient capitated fee for all related services. (Ms. Barnes pointed out that CMS will be conducting an experiment of a fully bundled rate for ESRD.)
- **Research implications.** An important consideration is that reimbursement policies influence pharmaceutical research priorities. These firms could be winners or losers if policies change, and need to be part of the conversation.
- **Immediate alternatives.** Because private plans already use specialty pharmacies, participants asked whether CMS could follow this lead. One idea was to allow private plans that offer Part D coverage to use their specialty pharmacies for enrolled beneficiaries, something that it not allowed today.

# Designing Physician Financial Incentives for Appropriate Care

Presenter: **Lee Blansett**, Senior Vice President, *MattsonJack DaVinci*

## Overview

"Buy and bill" reimbursement creates financial incentives that favor certain therapeutic choices. This has potential to place physicians' financial interests in conflict with patients' needs. Although these conflicts may exist, there is little systematic evidence that physicians make decisions to benefit financially at the expense of appropriate patient care.

One alternative to the current system is reducing drug margins (through shifting to average sales price reimbursement) while providing an increased administrative fees. A second alternative is paying for drugs on a cost basis, and implementing a management fee. These options face a variety of challenges including significant operational complexity.

## Context

Lee Blansett presented data on the profitability and treatment patterns under "buy and bill" reimbursement for selected specialty drugs, and offered an analysis of alternative reimbursement approaches for consideration. His presentation was followed by a participant discussion.

## Key Takeaways

- **"Buy and bill" has potential to create conflicts between physician and patient interests, but there is little systematic evidence that this is occurring.**

  Mr. Blansett posed the questions of whether "buy and bill" creates a potential conflict between physicians' financial interests and patients needs in regard to specialty drugs. Conflicts could arise if physicians were to choose the more profitable of two equal alternatives, choose a more profitable but less effective alternative, or over-treat.

  Mr. Blansett concluded that there is potential for conflict because office-administered specialty drugs generate positive margins for specialists in rheumatology, gastroenterology, and oncology. However, there is no evidence of this pervasively occurring.

  — *There is little systematic evidence of preference for the highest-profit alternative in rheumatoid arthritis.* An analysis of growth in the number of prescriptions of three rheumatoid arthritis drugs showed that the product with the highest physician profitability was growing the slowest.

  — *There is also little systematic evidence of physicians using lower-efficacy, higher-profit alternatives in cancer.* In research conducted by Mr. Blansett's firm, the most popular therapy for use in metastatic breast cancer was Xeloda, an expensive oral drug that provides no profit margin to most oncologists.

> *"There is little or no evidence that physicians are gaming the system, and allowing profit motives to conflict with patients' best interest.*
> — Lee Blansett

- **Physicians are taking patient financial status into account when selecting therapies.**

  There are situations where physicians are not prescribing drugs because patients are unable to pay; putting the patient and physician in conflict with insurers.

  In a study of 134 oncologists, 44% said they had not prescribed a cancer drug due to cost. This was rarely due to the cost to the provider (10%); in 64% of cases physicians believed that the cost to the patient was so high that the patient could not afford the co-payment. In such instances, physicians often prescribe a more costly Part B IV drug instead of an oral covered by Part D.

> *"The cost [to patients] of oral drugs covered under Part D encourages oncologists and patients to seek treatments under Part B."*
> — Lee Blansett

- **Alternatives to "buy and bill" should avoid perverse incentives, make physicians formulation agnostic, and be easy to implement.**

  It is not clear that developing alternatives to "buy and bill" is worth the effort. In 2006, Part B drugs only accounted for 2.6% of total Medicare spending. Still, projections indicate that specialty drug spending will grow significantly. Mr. Blansett believes many of these projections are over-stated because they assume most specialty drugs in development will be launched. History shows that only a fraction of them will come to market.

  If new approaches are considered, important goals include:

  — *Avoiding perverse incentives.* The incentives must be to provide the right treatment to the right patient at the right time.

  — *Keeping care in a community setting*. It is more cost-effective to deliver care in community-based offices or treatment centers than in hospitals. A poorly-designed policy may cause community physicians to cease offering services, and force care to more expensive hospital outpatient settings.

  — *Coordinating care*. There must be incentives to provide support and follow-up care to patients after they receive treatment.

  — *Making physicians "formulation agnostic."* Physicians should be financially indifferent to drug formulation, be it oral, infusible, or other. They should focus on what works best for a specific patient.

— *Leveraging information technology.* Implementing any new plan in a practical way requires using existing IT and organizational structures.

- **There are several alternative approaches to physician reimbursement that merit consideration.**

**Alternative #1: Maintain the status quo**

This means continuing to reimburse physician-administered drugs via "buy and bill."

— *Advantages*: Easy implementation; physician acceptance; and downward pressure on growth trends due to generics, follow-on biologics, and commercial plans switching from average wholesale price (AWP) to less profitable average sales price (ASP) contracts.

— *Disadvantages*: The cost growth trend will continue under the status quo; the current approach is driving practice consolidation; access for orals remains weak for many Part D patients; and physicians carry risk for non-payment (1–4% of revenues from infusible drugs are written off).

— *Other unintended consequences*:

- Medicare's ASP-based IV drug reimbursement policies have significantly lowered physicians' margins on chemotherapy treatments, and could cause some to lose money on them.
- As a consequence of lower reimbursement, some oncologists may be exiting the market. The result is that more patients are treated in costly hospital settings.

**Alternative #2: Getting docs out of the drug business**

This approach would adopt zero/low margin reimbursement policies on drugs, but increase fees paid for administration. Analysis shows that to maintain oncologists' incomes at ASP +6% would require that physician fees rise by about 200%.

— *Advantages*: Eliminating profit as a physician selection criterion for "buy and bill" drugs, and re-establishing equality of access for Medicare and commercial patients.

— *Disadvantages*: Operationally complex to implement when specialists like oncologists are part of an IPA. Also, reducing the margin per treatment could lead physicians to increase treatment volume. Physicians' inaccurate view of true drug costs may limit gains. And, while physicians lose profits from drug mark-ups, they retain the risk of non-payment.

— *Other unintended consequences*: Community oncology could adopt a conglomerate structure providing large chemotherapy centers with higher overhead and negotiating clout, versus treatment in individual physicians' offices; this trend has already begun.

**Alternative #3: Management/episode fees**

This approach would adopt episode fees to help replace income from drug mark-ups. The idea is to maintain the viability of community-based IV infusion through ASP +4-6% while adding a monthly management fee for cognitive services.

— *Advantages*: This would eliminate profit as a physician selection criterion for drug therapies. It would pay physicians for educating patients and managing care for both oral and IV drug therapies. It would reduce the attraction of conglomerate strategies.

— *Disadvantages*: This option is operationally complex for private payers to implement, and it is challenging to determine the terms and size of episode payments. There is also some risk for under-treatment. This means that payment per episode would require rigorous QA and UM systems to monitor under treatment.

## Participant Discussion

- **Physician compensation.** Jack Rowe advised participants to look at history to provide perspective on the various alternatives. Previously, oncologists were paid management fees and many abused this system. In Dr. Rowe's view, maintaining the physician income isn't a primary objective in changing the payment system for specialty drugs.

  Stuart Altman disagreed, saying that physician income is at the crux of this discussion. Policymakers will have to determine if "keeping physicians whole" is a policy objective; failing to keep physicians whole will incite strong resistance.

  Mr. Blansett noted that oncologists' incomes are already declining, down from $750,000 in 2004 to $300,000-$400,000 currently. Further declines could cause some to leave the profession. Dr. Rowe doesn't view this as a threat, as oncologists make far more than other physicians, and oncology is a desirable area of medicine filled with scientific advances.

  Don Moran suggested distinguishing between the practice of oncology as a profession and administration of chemotherapy as a freestanding commodity business. (This is analogous to the practice of nephrology and dialysis.) Oncologists made a great deal of money through freestanding chemotherapy businesses, but profits in these businesses are declining.

- **Paying for quality.** Participants commented on the lack of discussion regarding paying for use of protocols. The consensus was that doing so is desirable; however, currently difficult to implement.

- **Broader perspectives.** Beyond provider incentives, there are multiple factors that affect the total costs of specialty drugs. This includes the actual cost of the drugs (which is far more than the amount paid to providers to administer them), and use of drugs by patients who don't benefit from them.

Other factors that need examination include ways to lower drug costs and development of more targeted therapies. Kathy Buto suggested that pharmaceutical companies might receive longer exclusivity periods in exchange for lower prices. Other creative ideas were encouraged beyond just physicians' financial incentives.

# Designing Payment Policies to Promote Appropriate Utilization

Presenter: **Sharon Levine, MD**, Associate Executive Director, *Permanente Medical Group*
Respondent: **Newell McElwee, PharmD, MSPH**, Vice President, *Pfizer*

## Overview

Providing appropriate access to specialty pharmaceuticals while managing costs poses significant challenges for third party payers. There are several policy options for linking the price of these drugs to value (value-based pricing and evidence-based pricing). These options require evidence about which treatments are most appropriate for specific patient sub-populations. New payment policies must also try to establish incentives for continual development of more efficient, effective treatment options.

## Context

Dr. Levine discussed the relationship between appropriateness, value, and payment policy for specialty pharmaceuticals. She offered policy options to address these areas and discussed potential challenges that they would present. Dr. McElwee offered his perspective, and participants shared their views on these options.

## Key Takeaways

- **From a payer perspective appropriateness and value need to be incorporated in new specialty pharmaceutical payment models.**

  The key issues include:

  — *Value*: Defined as health benefit per dollar expended. There is currently no systematic mechanism for assessing the value of specialty drugs (or other high-priced medical, surgical, or pharmaceutical therapy). Dr. Levine disagreed with the statement that "once a potentially lifesaving therapy is developed, it is untenable for payers or society to limit access." This is due to the ambiguity inherent in the concept of the term "potentially lifesaving." Cost and access to care are tightly interrelated, but in our current system decision makers focus primarily on clinical effectiveness, not value.

  — *Appropriateness*. Determined by the physician as "the right drug for the right patient," based on safety and effectiveness. When more than one treatment option is available, relative cost effectiveness is a factor if that information is known. Patients' preferences also affect appropriateness, including decisions about dosing, duration, and administration.

  — *Payment policy*. Specialty drugs are extremely expensive, and have no generic equivalents. Tiered formularies that are effective for standard drugs aren't appropriate for specialty pharmaceuticals. The purpose of tiered formularies is to use the psychology of cost sharing to drive patients toward lower utilization and lower-priced products. But many specialty drugs lack alternatives and are not discretionary. This neutralizes the benefit of a formulary and simply limits access.

> *"The price of specialty drugs, not the psychology of payment, creates a barrier to access and appropriate use."*
> — Sharon Levine

- **Policy options for improving value and appropriateness of specialty drugs require a shift in how society thinks about medicine and tools for improving evidence.**

  To improve value, our society must confront the question of whether limits on drug pricing will stifle innovation. An increased focus on value will force new models of partnership between payers and manufacturers that keep price in the conversation.

  — *Appropriateness*. The key to appropriateness is having better information about which therapies are most appropriate for which patients. There are several options for gathering better information:

  - More robust pre-release safety data. This requires adequate funding for the FDA to conduct more robust pre-release safety review.
  - Coverage with evidence development. This would grant coverage but require evidence development as a condition of reimbursement.
  - Comparative effectiveness research. This would compare therapies within the same category to assess relative effectiveness and value.

  Other promising areas include greater use of data in electronic health records for appropriateness studies, and expanded research on using biomarkers to predict outcomes.

  — *Payment policy*. Several payment policy options exist:

  - Value-based pricing. The price of drugs would be based on clinical value, with a consensus indicator of value such as quality adjusted life years (QALY). Presently, there is no consensus measure in the U.S.
  - Evidence-based pricing. This would link product release prices to the strength of evidence of benefit at the time of release. Prices would be limited initially and adjusted upward as new evidence of positive benefit is produced. Pharmaceutical companies would have strong incentives to invest in evidence development post-FDA approval.
  - Reference pricing. Officials judge the therapeutic effectiveness of drugs within a disease group and reimburse based on the least expensive option. This is used in other countries, especially those with national health systems.
  - Co-insurance. Current models of patient coinsurance are problematic for specialty drugs because they are expensive and non-discretionary.

- A biogeneric regulatory framework with reasonable exclusivity. Four bills are in Congress to lengthen the period of exclusivity for pharmaceutical companies. This would give them more time to set prices in the absence of competition and recoup their investment. But delaying competitive products hinders innovation, as sequential innovation is driven by competition.
- Drug licenses. This idea incorporates two-part pricing for specialty drugs with a goal of increasing patient adherence. For example, a patient would pay his or her co-pay for 10 months but get 12 months of therapy. This could address the psychology of co-pays as an impediment to adherence, but not the economic impact of co-insurance.

- **Drug companies are grappling with the issues described. The challenge is balancing access and affordability.**

Responding to Dr. Levine's presentation, Dr. McElwee noted that these are the issues Pfizer grapples with each day. They make decisions in the context of two immutable truths: society must contain health care costs; and this must be done while balancing the fact that society wants more medical treatments.

> *"Doing one [cost containment or providing new medical treatment options] is easy, but we must do both. We need policies to balance patient access with payer needs for an affordable benefit."*
> — Newell McElwee

The challenge for the pharmaceutical industry is the long drug development lifecycle, which requires that companies think about prices five to seven years before releasing a drug. Pricing analysis involves calculations of expected net present value (NPV). Because resources are limited, NPV guides decisions on where and how much to invest. Companies stop development on some promising drugs due to a lack of upside potential.

Dr. McElwee responded to several specific issues raised by Dr. Levine:

— *Comparative effectiveness*. He supports comparative effectiveness, but believes it is not a panacea. Research results are often averages, and as such, are relevant to a given patient only to the extent that the patient is like the average.

— *Value-based pricing*. New measures other than cost (i.e. cost per QALY) have to be accepted in the U.S.

— *Evidence-based pricing*. He sees this as promising because it is analogous to using evidence to decrease uncertainty, which serves to increase value.

— *Reference pricing*. This will be a challenge for specialty pharmaceuticals in the United States.

— *Drug licensing*. He believes there could be many other models. For example, a Disney-like model might be considered where a patient pays one amount upfront to begin using a drug (like an admission fee at Disney) and then a small amount per use. This could better align incentives and increase appropriate use.

## Participant Discussion

- **Tying evidence to pricing.** Participants were interested in the idea of developing evidence after a drug's release and using it in pricing decisions. Today, manufacturers have few financial incentives to collect evidence after a drug's release because it doesn't affect pricing. Since gathering this evidence comes at a significant cost, and the rewards are few, it rarely occurs. Rob Mechanic posited that another reward for gathering evidence could be a longer exclusivity period.
- **How evidence affects innovation.** Participants had differing perspectives on whether linking price to evidence would spur or hinder innovation. Some thought it would diminish innovation; others thought that the potential for higher prices would cause companies to shoot for breakthroughs. One participant commented that there is already significant opportunity in specialty drugs. The industry is growing and attracting substantial capital. While it is important to consider new incentives, basic market conditions provide incentive enough for companies today.
- **Effect of "me too" drugs.** A question was raised about the value of "me too" drugs. Several participants responded that drugs are rarely "me too." Manufacturers attempt to develop drugs that are incrementally better, and offer different benefits to different patients. Multiple drugs in the same category also lead to price competition and broader treatment options.
- **Comparative effectiveness.** Responding to the remark that comparative effectiveness research is limited because it is based on averages, Dr. Levine commented that the goal of comparative effectiveness research is helping determine which patients are likely to benefit from a treatment. While not perfect, it can shift thinking from a focus of a "yes or no" to a "more or less appropriate" approval system.
- **Cost of failure.** Stuart Altman suggested that one reason why the price of specialty drugs is so high is because few drugs in the pipeline actually get to market. Those that do are expected to generate enormous returns to offset the many costly failures. If the costs of failing could be mitigated, perhaps pricing on the successes could be reduced.
- **Barriers to paying for value.** If the concept of paying based on value is valid, why don't private payers move in this direction? The answer for many specialty drugs: there are no alternative therapies, and payers lack leverage to pay based on value. In addition, it is difficult to measure and quantify value.
- **Why co-pays/co-insurance for specialty drugs?** When asked why insurers created Tier 4 plans when even they don't particularly support them, Dr. Levine and others pointed out that employers allocate a limited pool of money for healthcare coverage, but demand a specialty drug benefit. When they say, "Figure out how to do this," the result has been the flawed benefit structure for specialty drugs that exists today.

# Addressing Social Tradeoffs of Copayments for Specialty Pharmaceuticals

Presenter: **Don Moran**, President, *The Moran Company*
Respondent: **Amy Melnick**, Chief Public Policy Officer, *The Arthritis Foundation*

## Overview

Tier 4 benefit design for specialty pharmaceuticals creates socially untenable consequences. It is essentially a cruel lottery where beneficiaries with the misfortune to be diagnosed with a rare disease that requires costly specialty drugs must bear the financial burden. Health plans are unable to individually eliminate Tier 4 benefit design because they would face immediate adverse selection if their competitors did not follow suit. Potential policy solutions include local legislation that would ban Tier 4 policies and government sponsored reinsurance programs that would spread the risk to a larger pool of beneficiaries.

## Context

Don Moran described the issues associated with Tier 4 benefit designs, and offered options for mitigating the current situation. Amy Melnick shared the perspective of The Arthritis Foundation, a patient advocacy organization, and participants commented on the policy options laid out by Mr. Moran.

## Key Takeaways

- **Tier 4 benefit design was a natural evolution from the tiered drug benefits of the past 25 years.**

  Over the past quarter century, tiering has become universally loved in the benefits management industry. It began with a base tier (Tier 1) for generic substitutes while Tiers 2 and 3 enforced favorable rebates on the branded side. When very high cost drugs began appearing earlier this decade, a fourth tier co-pay scheme seemed like a natural evolution of prior policy.

  The Tier 4 concept wasn't generated by health plans. About six or seven years ago, benefits consultants began inserting requests for a fourth tier in benefits RFPs; plans simply responded to these requests. Plans also saw tiering as a palatable alternative to command-and-control policies. Instead of denying access to drugs, plans simply put high prices on them.

  > *"Why take the heat for denying drugs to patients when you can just let them talk themselves out of it [because the price is so high]?"*
  > — Don Moran

  In addition, given the high rate of off-label use for some drugs, the fourth tier provided plans with some form of protection from runaway costs. Over the past decade, the Tier 4 model has become ubiquitous in the industry.

- **Tier 4 benefit design is essentially a structured game of Russian roulette—a huge problem for society.**

  Insurance is designed to help smooth the financing of low-frequency, high-cost events. Everyone contributes a small amount to an insurance pool which then pays claims for those experiencing the low-frequency, high-cost events.

  But the Tier 4 design "de-pools" the risk back to each beneficiary. Beneficiaries then participate in a structured game of Russian roulette. Because they bear a large portion of the risk, the unfortunate individuals that experience low-frequency, high-cost events are exposed to devastating financial consequences. Society's failure to deal with this indicates a policy that has gone terribly wrong.

  > *"Those who lose this lottery lose everything."*
  > — Don Moran

  Ms. Melnick agreed with Mr. Moran's characterization of low-probability events as a lottery; those who get rheumatoid arthritis, through no fault of their own, have been selected through a cruel twist of faith.

  There are more than two million people in the United States with rheumatoid arthritis, Ms. Melnick reported. It is an unavoidable disease with no known causes which tends to strike middle age women. Most who get this disease benefit from drugs, which are expensive, and usually taken on an ongoing basis. But only about 50% of patients get and take the drugs they need. Many can't afford their medications, which has a great impact on rates and duration of disability.

  Unfortunately, health plans feel as though they are stuck and can't move away from the current benefit structure. The first plan to offer a drug benefit without a fourth tier would experience profound adverse selection as every high-cost patient would sign up. As a result, no plan will be the first mover. To deal with this problem, something must change.

- **Options to deal with this problem include regulation and reinsurance.**

  Large employers have begun to observe that the current benefit design is randomly producing bankruptcies among employees, which is not a favorable outcome. These employers can take action by creating a risk pool for their own employees for a small amount per member per month. However, the situation is unchanged for private insurers.

This appears to be a situation where a government intervention may be appropriate. There are two types of interventions that might be effective:

— *Federal regulation*. This would entail regulating the specifics of fourth tier benefit designs, detailing what plans can and can't offer. This would be difficult because insurance is regulated by states and there is no practical way for federal regulators to insert themselves.

— *Reinsurance*. A federally supported program could reinsure the risk for carriers willing to eliminate the fourth tier. The model could be a free-standing federal program or subsidies for private insurers. One complexity would be defining what constituted the risks that were covered by reinsurance. Also, larger employers would probably want to participate, substantially increasing the cost.

## Participant Discussion

- **Reinsurance.** Senators Obama and McCain have both expressed support for a federal reinsurance system. A reinsurance system could focus on specialty pharmaceuticals, or cover all high-cost therapies. Mr. Moran pointed out that the more ambitious the goal, the longer it would take for action.
- **Local bans.** Since insurance regulations are state-based, advocates could adopt a state by state effort to ban Tier 4. This was recently attempted in one state but did not pass. Banning in an entire state would keep high cost patients from moving to plans that eliminated Tier 4 individually.
- **Using evidence to make tier decisions.** One participant suggested an alternative to the current tier process. While today drugs are assigned to the third or fourth tier based solely on expense, his idea is a form of "evidence-based tier system." When drugs are used on-label and are supported by sound evidence, these should be covered as part of Tier 3. But for drugs used off-label or where a drug lacks appropriate evidence, the drug is in Tier 4. If patients want a Tier 4 drug, despite the lack of evidence, they can have access to it but must pay the hefty co-insurance. Mr. Moran thought that this would be extremely difficult to implement.
- **Focusing on catastrophic coverage.** Current policies cover typical day-to-day medical expenses, yet often fail to cover catastrophic expenses. If patients were asked what they really want, perhaps coverage for routine expenses would be less important than protection against catastrophic expenses.
- **Patient assistance programs.** Most pharmaceutical companies have patient assistance programs to "fill gaps." They provide drugs for low-income patients in need, help with co-pays, and fill in the donut hole for low income Medicare beneficiaries. Like financial aid programs at universities, these programs enable a form of price discrimination. However, patient assistance program, although helpful, do not fully address the scope of necessary financial assistance.