

**July 16, 2008**

## Specialty Pharmaceuticals: Balancing Access, Affordability, and Incentives for Innovation

The growing availability of specialty pharmaceuticals represents an emerging challenge for domestic health policy. These drugs, typically biologics and advanced chemotherapeutic agents, can cost tens of thousands of dollars per treatment, drawing the attention of third party payers who seek to manage their use. While these products have sometimes demonstrated miraculous results in clinical trials, it is often difficult to identify which patients will benefit from specific therapies. Specialty pharmaceutical costs are growing at 15-20 percent annually. Although used by only a small percentage of patients, specialty drugs now represent about 20 percent of total outpatient pharmaceutical spending. The large number of specialty pharmaceuticals and genome-based therapies currently in clinical development are certain to exacerbate these trends.

To limit the economic burden, payers are trying to curb off-label and low-value uses, and reform the distribution system in which physicians can earn sizable profits by purchasing specialty drugs and billing insurers at significant mark-ups. They are also increasing patient cost sharing. Some plans, including many of those serving Medicare beneficiaries, have added a "Tier 4" to their formularies for specialty drugs, under which beneficiaries typically must pay 25-33 percent co-insurance. As a result, patients may face significant financial barriers as they decide whether to initiate potentially life-saving treatments.

On July 16, 2008, The Health Industry Forum hosted a conference entitled, "Managing Specialty Pharmaceuticals: Balancing Access and Affordability." Participants from academia, government, health plans, delivery systems, biopharmaceutical firms, and consumer groups met to discuss the specialty market, examine health plan strategies for managing specialty products, and identify key issues for Medicare. The meeting also examined more fundamental questions about how much society should be willing to pay for very expensive treatments, and how these costs should be distributed among both the sick and the well. Key themes are summarized below.

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### *Payers have adopted a range of strategies for managing specialty pharmaceuticals.*

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As with other expensive therapies, payers have applied a host of traditional strategies to try and ensure that the right drug is targeted to the right patient, including clinical guidelines, prior-authorization, step-therapy, and other utilization management techniques. Because of the high unit cost of specialty pharmaceuticals, insurers are trying to reduce waste by limiting the duration of initial prescriptions, re-evaluating medical necessity based on patient response, and restricting reimbursement for off-label uses. Additionally, in an effort to better control use and lower acquisition costs, insurers are increasingly using specialty pharmacies to consolidate product distribution and manage expensive therapies more efficiently.

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### *Efforts to promote appropriate use of specialty pharmaceuticals are hampered by a complex benefit structure, misaligned incentives, and limited outcomes data.*

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1. ***Overlapping medical and pharmaceutical benefit plans with different rules and payment rates.*** Many specialty pharmaceuticals must be injected or infused in a physician office or clinic. A key issue in benefit design is whether a physician-administered specialty drug should be considered a medical service or a prescription. Historically, physician-administered drugs have been paid for through the medical benefit as an overhead supply of the practice. Thus, physicians purchase the drugs and submit claims through a process known as "buy-and-bill."

Because drug coding protocols for medical claims are imprecise, payers' have difficulty monitoring specialty pharmaceutical utilization. Medicare and private insurance companies are increasingly shifting drugs from the medical to the pharmacy benefit in order to monitor and manage drug utilization more effectively. However, as drugs are shifted, they are subject to different rules, payment rates, and beneficiary coinsurance.

2. ***Misaligned physician financial incentives.*** Buy-and-bill reimbursement increases spending because physician claims frequently include large mark-ups. For example, oncologists are reported to receive more than half of their practice revenues from drugs. However, for many clinical indications some drugs are covered under the pharmacy benefit while others are in the medical benefit. In these instances, physicians have financial incentives to recommend products in the medical benefit, even if a more appropriate choice is available through the pharmacy benefit.
3. ***Lack of systematic data on patient outcomes.*** Targeting the right drug to the right patient requires a system for documenting what works and what doesn't for patients with different clinical characteristics. Within labeled indications, this is predominantly accomplished through clinical trials. But specialty pharmaceuticals are heavily prescribed for off-label indications and for populations not studied in trials. The great (but often unproven) potential of these products places tremendous pressure on insurers who must balance overall costs against the demands of seriously ill patients in the face of limited outcomes data.
4. ***Increased patient co-insurance.*** Most controversially, over 85 percent of all Medicare Part D drug plans have instituted four-tier formularies, in which patients must pay 25-33% of the cost of high-priced drugs listed in the top tier. Under Part D, Medicare beneficiaries have some protection because Medicare catastrophic coverage rules limit coinsurance once patients have reached annual out-of-pocket spending of approximately \$4,400. Commercial health plans also have implemented Tier 4 policies in about thirty percent of offerings; unlike Medicare Part D, these programs may not include catastrophic protection. High coinsurance creates agonizing choices for patients about whether to initiate treatment and degrades the effectiveness of chronic care management if patients start and stop therapy based on their ability to pay.

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***New public policies are needed to achieve a better balance of access, affordability, and incentives for future innovation.***

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Public and private policies could promote more appropriate use of specialty pharmaceuticals. Potential solutions should focus on four areas:

1. ***Creating a more integrated infrastructure for managing specialty pharmaceuticals.*** A more integrated benefit structure for specialty pharmaceuticals could help create more consistent financial incentives and facilitate clinical management. This is particularly important for Medicare which, up to this point has not used many of the clinical management strategies common in the private sector. For example, Medicare does not now have a formulary for Part B drugs or contract with specialty pharmacies to manage complex therapies. It is conceivable to envision a separate benefit devoted solely to specialty pharmaceuticals, although, this would require significant policy changes.
2. ***Establishing balanced physician financial incentives across therapeutic choices.*** Payers and policymakers are interested in exploring alternatives to the buy-and-bill system that would result in neutral prescribing incentives. A revised system that reduces income from drug mark-ups would need to increase fees for patient management and drug administration sufficiently to ensure that patients receive appropriate levels of service. Such changes will be challenging to implement to the extent that some specialty practices would face significant losses in income. Payers also do not want changes that will shift patient care from physician offices to more expensive hospital and clinic settings.

3. **Enhancing evidence of effectiveness for specialty pharmaceuticals, especially for off-label uses.** Clinical development of biopharmaceuticals does not end once the product is FDA approved. More systematic data collection through post-marketing studies, national registries and coverage with evidence development (CED) could provide more credible, comprehensive information about how products are being used, their safety and effectiveness, and how outcomes vary across sub-populations. A key issue, however, is determining the sources of funding for this ongoing research. In addition, there is significant interest in companion diagnostics that would help determine which patients are appropriate candidates for expensive therapies, though few such diagnostics are currently available.
4. **Resolving the financial burden of Tier 4 co-insurance.** To many, Tier 4 policies contradict the basic premise of insurance: providing financial protection against the catastrophic costs of serious illness. Furthermore, some question significant cost sharing for specialty drugs, when the same policies do not apply to other expensive services like surgery. Health plans argue that covering new specialty pharmaceuticals without effective mechanisms to assure appropriate use substantially raises premiums. Plans that do not use four-tier formularies when their competitors do risk serious adverse selection as high-cost patients would have strong incentives to select their products. There is some interest in state or national policies that would restrict Tier 4 co-insurance levels or require income-adjusted out-of-pocket maximums. Alternatively, the federal government could create a re-insurance program with case management for very high-cost patients.

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***How these issues are addressed for specialty pharmaceuticals may foretell how nation policy will deal with growing conflicts between expanding medical progress and narrowing health insurance benefits.***

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While specialty pharmaceuticals represent only 2-3 percent of total health spending, this sector embodies trends that are evident across the rest of the system: rapidly rising costs, insufficient evidence of effectiveness, increased patient cost sharing, and misaligned provider financial incentives. The high cost of specialty pharmaceuticals leads to questions about the value of specific interventions and whether there is a limit on what society can afford. Unlike their European counterparts, US policymakers have been hesitant to discuss whether some beneficial therapies should not be covered because their costs significantly exceed their benefits.

The growing controversy over Tier 4 formularies also raises questions about how we equitably share the burden of illness between the sick and the well. If the specialty pharmaceutical cost trend continues to grow, insurers and pharmaceutical companies risk a consumer backlash against the high prices and the failure of insurance to adequately protect patients. Public pressure for action to address high-cost therapies could lead to a fundamentally different role for government. As these trends develop, the need to find a workable balance between access, affordability, and innovation will become increasingly urgent.

***Note: The Forum held a follow up specialty pharmaceuticals meeting on October 2, 2008 to discuss policy solutions for the issues addressed in this document. That discussion is summarized in a companion policy brief and report available on the Forum's website.***

*This policy brief was prepared by Darren Zinner and Robert Mechanic of Brandeis University Conference presentations and a more detailed proceedings document are available at [www.healthindustryforum.org](http://www.healthindustryforum.org)*

***The Health Industry Forum*** is based at Brandeis University and chaired by Professor Stuart Altman. 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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