

THE GROWTH OF SPECIALTY PHARMACY

Current trends and future opportunities

Issue Brief
April 2014

Summary

Innovative specialty drugs are providing important cures and treatments, with new therapies expected in the near future. However, annual costs for certain drugs may reach \$30,000 and in some cases exceed \$100,000.^{1,2} Already these drugs comprise over a quarter of total drug spending in the United States and spending is growing at double-digit rates even as the growth rate for traditional pharmaceuticals has slowed.³ This Issue Brief contains new data from UnitedHealth Group on current trends; examines the opportunities and challenges presented by these new therapies; and outlines various solutions including modernizing payment policy, adopting new modes of clinical management and improving data analytics.

Key points:

- Patients using specialty drugs have complex conditions and care needs that require support and coordination; costs and access are growing challenges. **Spending on specialty drugs in 2012 in the United States was about \$87 billion. Estimates suggest that it could quadruple by 2020, reaching about \$400 billion,** or 9.1 percent of national health spending. Unit price growth is driving spending increases but utilization growth plays a strong role for certain therapies.
- **About half of spending for specialty drugs is funded as a pharmacy benefit; the other half is funded as a medical benefit,** leading to challenges in integrated clinical management.
- Analysis of 2012 claims data from UnitedHealthcare's commercial members shows that about **51 percent of spending on specialty drugs is for cancer, rheumatoid arthritis, and multiple sclerosis.** In the Medicare program, oncology drugs comprise about 36 percent of spending in the specialty area with drugs that treat end-stage renal disease (ESRD) and cardiovascular conditions making up another 16 percent of spending. In Medicaid health plans, HIV drugs account for a significant share of spending on specialty pharmacy (about 18 percent). It will be important to pursue targeted strategies that focus on treatment for those conditions and their related medical care.
- Specialty drugs particularly impact **Medicare beneficiaries,** who have relatively high spending (on a per person basis) for those drugs, about double the amount of spending by commercial health plan members.
- **Misaligned payment incentives are leading to provision of drugs in high-cost settings** of care. For example, UnitedHealthcare finds that per member per month costs for injectable oncology drugs in outpatient hospital settings are about 30 percent higher than costs in physician office settings. Strategies that more appropriately align incentives can help patients get the right drug in the right setting.
- Greater use of **coordination and adherence programs,** of the kind provided by specialty pharmacies, shows particular promise in improving outcomes and reducing costs. UnitedHealth Group research has demonstrated that specialty pharmacy and synchronized medical and pharmacy services yielded total cost savings of about 13 percent for cancer and transplant services and increased compliance for patients with cancer, multiple sclerosis, and rheumatoid arthritis. Single points of patient contact and connections to related services may help improve the care experience.
- Other approaches include adoption of **new payment models, better information on treatments and outcomes, development of patient registries, clinical pathways, and new forms of collaboration** between patients and providers.

What makes specialty drugs “special?”

Treatment for complex or life-threatening health conditions now includes the use of certain drugs broadly referred to as specialty drugs. These are typically made using advanced biotechnology methods and are referred to as “biologics” or “large molecules.”⁴ While no standard definition exists, specialty drugs generally are defined as having one or more of the following characteristics:

- Complex to manufacture, requiring special handling and administration
- Injectable or oral, self-administered or administered by a health care provider
- Costly, both in total and on a per-patient basis; taken by a relatively small share of the population who have complex medical conditions
- Difficult for patients to take without ongoing clinical support; also challenging for providers to manage⁵

A decade ago, specialty drugs were commonly referred to as “injectable drugs” and were used to treat conditions like cancer, rheumatoid arthritis, multiple sclerosis, and growth disorders; today, their use has expanded beyond those conditions to include treatment for other chronic and inflammatory conditions and through other modes of administration. The Food and Drug Administration (FDA) has approved about 300 drugs, which many industry stakeholders consider “specialty,” compared to a mere handful available two decades ago.⁶

Spending and utilization

Spending on specialty drugs in 2012 in the United States was about \$87 billion, comprising roughly 25 percent of total drug spending (up from 20 percent in 2010) and representing about 3.1 percent of national health spending.^{7,8,9} In the commercial sector, specialty drugs account for about 30 percent of total drug spending.¹⁰ In the Medicare Part D program, specialty drugs represent a smaller share of total spending (about 16 percent), reflecting higher traditional drug utilization and spending more generally in the senior population.¹¹

Spending growth. Cost trends for specialty pharmacy have steadily increased since 2000, even as the growth in costs for

traditional drugs has slowed (due to factors such as patent expirations and generic substitution).¹² Recent research suggests that per capita growth for specialty drugs is in the double-digits:

- One analysis shows per capita growth rates ranging from 14 percent to 20 percent a year for specialty drugs in the commercial market for the three largest pharmacy benefit managers.¹³
- Estimates for near-term cost growth suggest those trends will continue, ranging from 13 percent to 25 percent per-member-per-year for all payers.¹⁴

Although there are a range of possible trajectories for future growth in specialty spending, estimates suggest it could quadruple by 2020, reaching about \$400 billion, or 9.1 percent of national health spending.¹⁵ The composition of the pharmaceutical and biologic research and the development pipeline underscores this future growth. About 40 percent of drugs under development (about 650) are considered specialty drugs (close to half of these are expected to be used to treat cancer).¹⁶ And at least 60 percent of new drugs expected to be approved for marketing in the United States in the near term will be specialty drugs.¹⁷

The spending implications are substantial. About 65 percent of spending on new drugs over the last two years was for specialty drugs.¹⁸ Over half of the specialty drugs in the pipeline are high-cost oral medications, which are increasingly being developed to substitute for other types of treatments, such as injectable drugs provided in physician offices, outpatient settings or infusion centers.¹⁹ Within four years, industry estimates suggest that seven out of 10 of the top-selling drugs in the United States will be specialty drugs.²⁰

Costs for patients. A relatively small share of the population uses specialty drugs—in the commercial population, approximately three to four of every 100 plan enrollees use at least one.^{21,22} Therefore, per-person specialty drug costs are high, ranging from several thousand dollars to hundreds of thousands of dollars annually for some of the highest-cost products. Individuals often have substantial cost-sharing associated with those drugs. They also tend

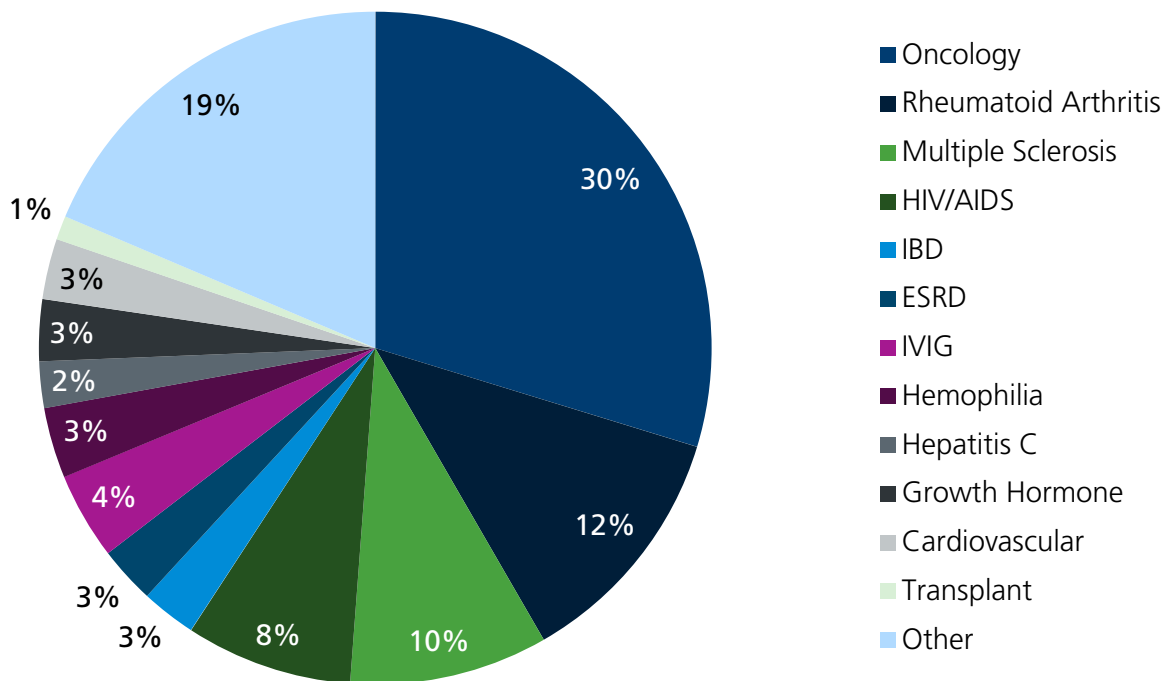
to have relatively high health care costs and account for about a quarter of health spending in the commercial population.^{23,24} For individuals who take them, spending on specialty drugs constitutes about 30 percent of drug and medical costs combined, while spending on other drugs and medical services related to their conditions accounts for another 27 percent.²⁵ People with several chronic conditions, like multiple sclerosis and rheumatoid arthritis, rely heavily on those drugs in their treatment. Over half of the total cost of care for many chronic conditions is attributable to specialty drugs.²⁶

Differences by payer. Medicare beneficiaries are more likely to use specialty drugs than younger populations. One study showed that compared to spending in the commercial population, spending on specialty drugs for Medicare beneficiaries is almost double the amount on a per-member-per-year basis while spending is about half the amount in the Medicaid population (under the pharmacy benefit).²⁷ Analysis of UnitedHealthcare claims data of spending on specialty drugs provided under the medical and pharmacy benefit combined had similar findings. Spending for specialty drugs

in 2012 for commercial health plan members was about \$39 per-member-per-month; per-member-per-month spending for Medicaid health plan enrollees was about half that amount, while estimated spending for Medicare beneficiaries was about double that amount.²⁸

Differences by category of therapy. UnitedHealth Group analyzed spending and utilization for specialty pharmacy drugs by therapy class using an internally developed approach that relies on claims data. Analysis of 2012 claims data from UnitedHealthcare’s commercial members, which includes spending for its members through pharmacy and medical benefits, shows that about 51 percent of spending on specialty drugs is for cancer, rheumatoid arthritis, and multiple sclerosis. (See Figure 1). Cancer drugs comprise about 30 percent of spending, most of which is for chemotherapy drugs. UnitedHealthcare’s experience is consistent with other industry estimates for commercial plans.^{29,30} Oncology also represents the dominant therapeutic area in UnitedHealthcare’s Medicare (36 percent) and Medicaid health plan (22 percent) populations.

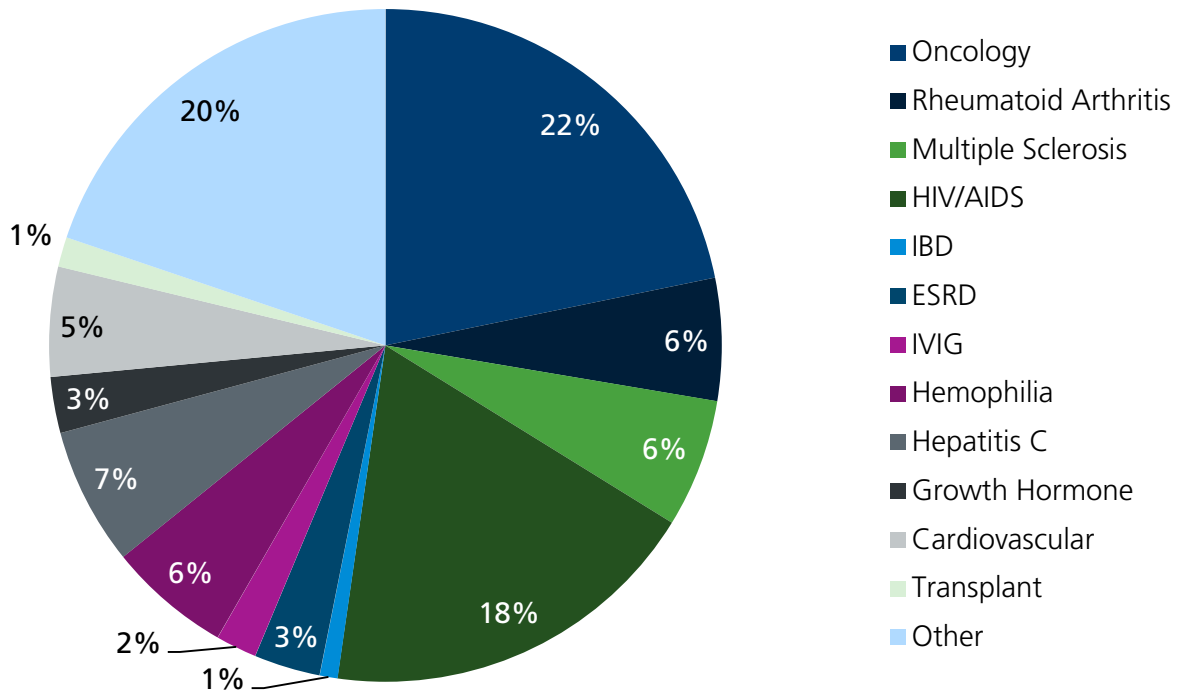
Figure 1; Share of specialty drug spending by disease state for UnitedHealthcare fully insured commercial plans, 2012



Source: UnitedHealth Group, 2014

Notes: Includes spending under the pharmacy and medical benefit; IBD = inflammatory bowel disease, ESRD = end-stage renal disease, IVIG = intravenous immunoglobulin. Shares do not sum to 100 percent due to rounding.

Figure 2; Share of specialty drug spending by disease state for UnitedHealthcare Medicaid health plans, 2012



Source: UnitedHealth Group, 2014

Notes: Includes spending under the pharmacy and medical benefit; IBD = inflammatory bowel disease, ESRD = end-stage renal disease, IVIG = intravenous immunoglobulin

The distribution of spending varies by payer, reflecting the conditions affecting different populations. In UnitedHealthcare’s Medicaid health plans, drugs that treat cancer, multiple sclerosis, and rheumatoid arthritis make up only 34 percent of spending. Drugs that treat HIV and hepatitis C comprise another 25 percent of spending. (See Figure 2.)

Handling and distribution

Because of specialized requirements for handling, delivery, storage, and preparation prior to administration, the distribution of specialty drugs often demands skilled management. As a result, the FDA requires manufacturers to adopt certain practices in this area for distribution of those drugs and manufacturers look to specialty distributors to help deliver their products.³¹ The dynamics of the supply chain also have changed with companies called specialty pharmacies evolving to address the complexity associated with specialty drugs. Specialty pharmacies have the

enhanced capabilities and infrastructure these drugs require and can support complex distribution and patient support services. These companies are playing an increasing role in the marketplace; though they may operate independently, they more often are owned by other entities such as pharmacy benefit managers (PBMs), retail pharmacy chains, wholesalers or health plans. Although traditional pharmacies can dispense specialty drugs, manufacturers may provide some drugs exclusively to specialty pharmacies. For drugs provided under the medical benefit, providers and patients also may rely on specialty pharmacies to access those drugs (though may also do so directly with manufacturers).

Administration and use in care

Compared to traditional drugs, patients often require the assistance of health professionals as they receive their specialty drug therapies. This assistance most often takes one of three forms: 1) patients may receive intravenous infusions or injections at physician offices; 2) those with chronic

conditions or undergoing chemotherapy treatment may visit infusion centers for ongoing therapy; and 3) patients who are taking self-injectable, oral or inhalable medications or those using skin patches may need concurrent treatment in physician offices, outpatient hospitals, other ambulatory care settings, or their homes (with the assistance of nurses or case managers). Specialty drugs often have specific regimen and dosage requirements that require provider oversight or assistance. For example, oncology drug regimens are particularly complex; patients can take multiple specialty and other drugs several times per day to treat cancer, address the side effects, and improve patient immune systems. Those cancer drugs, including oral oncology drugs, can have severe and life-threatening side effects. They require careful monitoring to ensure drugs are working effectively, that patients are taking appropriate doses, and that side effects do not lead to adverse outcomes.

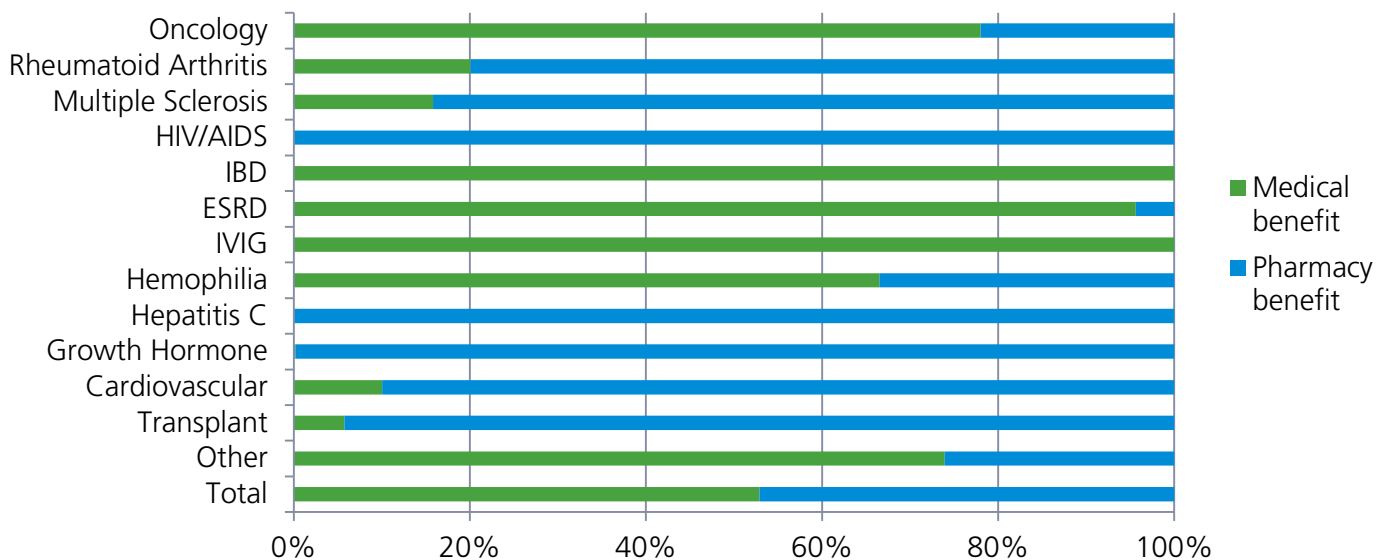
Benefit structure

When patients administer specialty drugs themselves or take them orally, the *pharmacy benefit* associated with their health plan generally provides coverage, often through the use of a PBM. In contrast, when a health care provider

administers specialty drug therapies, payers tend to cover the drugs and the related therapy as part of a medical benefit, which has a different approach to payment. Physicians administering intravenous specialty pharmaceuticals under the *medical benefit* purchase the drugs and then seek reimbursement from payers in a process called “buy and bill.” Hospital outpatient facilities also purchase those drugs themselves, negotiating their own arrangements with manufacturers, and bill payers for the drugs as part of the overall service. Slightly more than half of total spending on specialty drugs across all payers is for drugs covered under the pharmacy benefit: \$39 billion in medical benefit drugs, and \$48 billion in pharmacy benefit drugs in 2012.^{32,33,34}

When spending is broken down by clinical condition, a more complex picture emerges. For some conditions—for example, multiple sclerosis or growth deficiency—most specialty drug spending falls under the pharmacy benefit. That means that patients using those drugs tend to self-administer and PBMs/specialty pharmacies negotiate the drug price. For most cancers, the opposite is true, with most spending covered under the medical benefit and providers playing a role in drug acquisition. (See Figure 3 for a breakdown of spending by benefit category in UnitedHealthcare’s commercial membership and Appendix B for the same breakdown under

Figure 3; Distribution of spending for specialty drugs by type of benefit and disease state, UnitedHealthcare fully insured commercial plans, 2012



Source: UnitedHealth Group, 2014

Notes: IBD = inflammatory bowel disease, ESRD = end-stage renal disease, IVIG = intravenous immunoglobulin

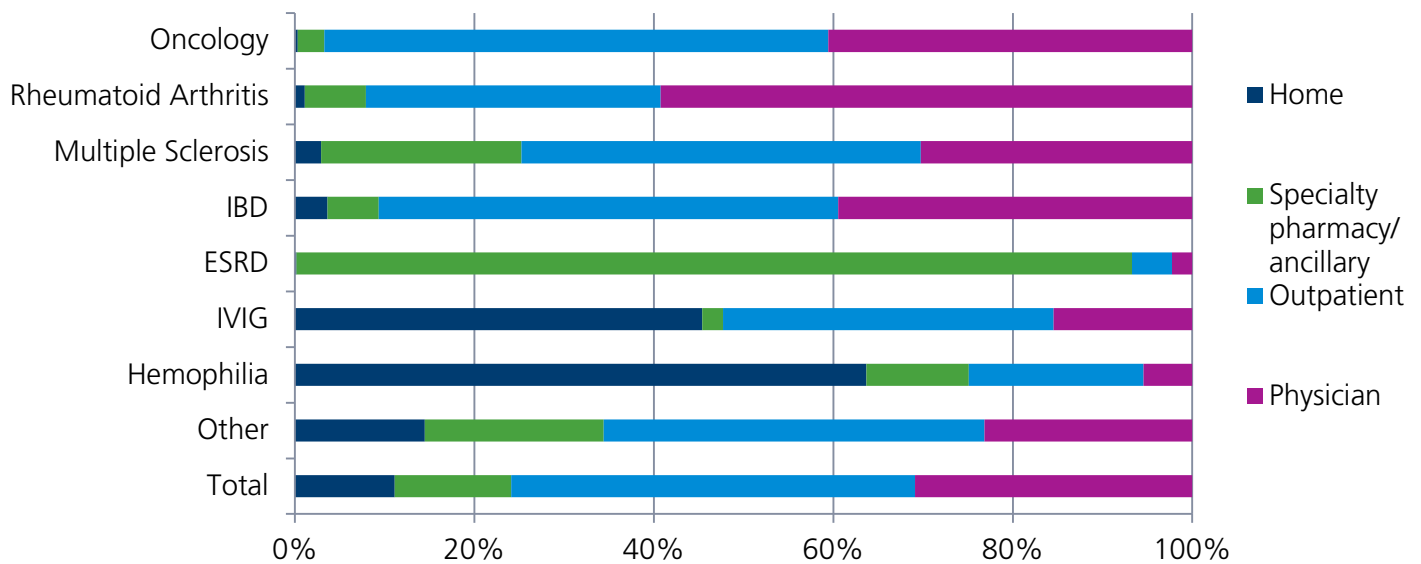
UnitedHealthcare's Medicaid health plans.) This explains in part why a substantial share of specialty drug spending under the medical benefit is for oncology drugs.^{35,36}

Understanding the distinctions between medical and pharmacy benefit coverage is important as it impacts approaches for managing patient care, provider networks, and reimbursement. For example, Hepatitis C and HIV drugs are primarily covered under the pharmacy benefit, while intravenous immunoglobulin (IVIG) and end-stage renal disease (ESRD) drugs are mainly provided as part of the medical benefit (see Figure 4). And for some conditions, patients may have options to take drugs covered under either benefit approach, creating multiple avenues for treatment and sites of care. Therefore, there is no single management strategy to address all specialty drugs.

In UnitedHealthcare's commercial population, 53 percent of specialty drug spending occurred under the medical benefit in 2012; by contrast, in UnitedHealthcare's managed Medicaid population, one-third of specialty drug spending was covered under the medical benefit and two-thirds under the pharmacy benefit. This differential is mainly due to the relatively higher spending for HIV drugs in the Medicaid population.

Within the medical benefit for the commercial population, about half the specialty drug costs are incurred in hospital outpatient facilities; however, patients access these injectable or infused drugs in other settings as well, including their homes (see Figure 4). Similarly, in UnitedHealthcare's Medicare and Medicaid health plan populations, hospital outpatient facilities are the primary setting of care for administration of specialty drugs under the medical benefit.

Figure 4; Distribution of spending for specialty drugs by setting of care and disease state, medical benefit, UnitedHealthcare fully insured commercial health plans, 2012



Source: UnitedHealth Group, 2014

Notes: Includes therapy categories which represent more than 10 percent of spending on total specialty drugs; IBD = inflammatory bowel disease, ESRD = end-stage renal disease, IVIG = intravenous immunoglobulin

Challenges to the affordability and quality of care

The high—and increasing—cost of specialty drugs represents a challenge for individual patients as well as federal and state budgets and private payers and raises important questions about the overall affordability of health care. Ensuring the most effective treatment approaches and best outcomes for patients taking specialty drugs can prove difficult. Current challenges in addressing those concerns include the following:

- Spending growth pressures
- Misaligned incentives that drive higher overall costs
- Barriers to effective drug utilization management
- Issues with treatment adherence and care coordination
- Gaps in data on treatment and utilization
- Limited evidence on effectiveness, including comparative effectiveness research

Spending growth and the role of unit prices

Both increases in utilization and unit prices contribute to spending growth for specialty drugs. However, unit prices have been the primary driver of cost growth overall in recent years and continue to escalate. In the case of drugs purchased under the pharmacy benefit, unit price increases between 2011 and 2012 accounted for almost all growth in per-member-per-year costs for specialty drugs in commercial health plans, Medicare Part D, and Medicaid (18.7 percent, 26.8 percent, and 16.7 percent, respectively).³⁷

Utilization growth also drives spending increases. The reasons are multi-fold, including: the prevalence of chronic conditions, the number of available treatments, new uses for existing drugs, and increasing cure rates/patient life spans. Those factors contribute to higher demand and use of specialty drugs.³⁸ For example:

- In the commercially insured population, annual per-member growth in utilization was 9 percent (compared to 14 percent for unit prices) for drugs that treat rheumatoid arthritis between 2011 and 2012.
- In the Medicare Part D program, utilization for drugs to treat many conditions, including cancer, multiple

sclerosis, and rheumatoid arthritis grew at rates ranging from 7 percent to 12 percent between 2011 and 2012.

- In the Medicaid program, utilization growth kept pace with unit price growth for drugs that treat inflammatory conditions, pulmonary hypertension, and respiratory conditions.³⁹

Price pressures represent a particularly difficult challenge in the specialty drug arena, where high unit prices reflect the costs of research and development and of bringing to market drugs that treat a small population (these drugs have higher unit prices as the costs of their development are spread over fewer users).⁴⁰ From a manufacturer's perspective, there is a need to charge prices that are high enough to cover their development costs, provide a return on investment, and have continued incentives for innovation.

In the United States, high prices also reflect regulatory policy that enables manufacturers to maximize revenue during a period of market exclusivity.⁴¹ High rates of growth in prices reflect the introduction of new and often expensive products, such as oral chemotherapy drugs. New FDA approaches for break-through drugs are likely to accelerate approvals of new products.⁴²

Private and public payers have limited ability to drive price discounts in this environment, which can lead to higher spending on those drugs. Unlike traditional pharmaceuticals, most specialty drugs have few (or no) clinically equivalent substitutes. Given the combination of limited or no substitutes and required coverage, payers are constrained in their ability to negotiate price concessions. Payers also have less negotiating power for specialty drugs provided under the medical benefit, particularly in areas with a high level of provider concentration. Even so, new therapies for rare conditions are carrying prices that may be difficult to sustain.⁴³

Market dynamics and incentives that may lead to high costs of care

Incentives in the current system may drive higher spending for specialty drugs than may be appropriate, particularly for drugs provided under the medical benefit.

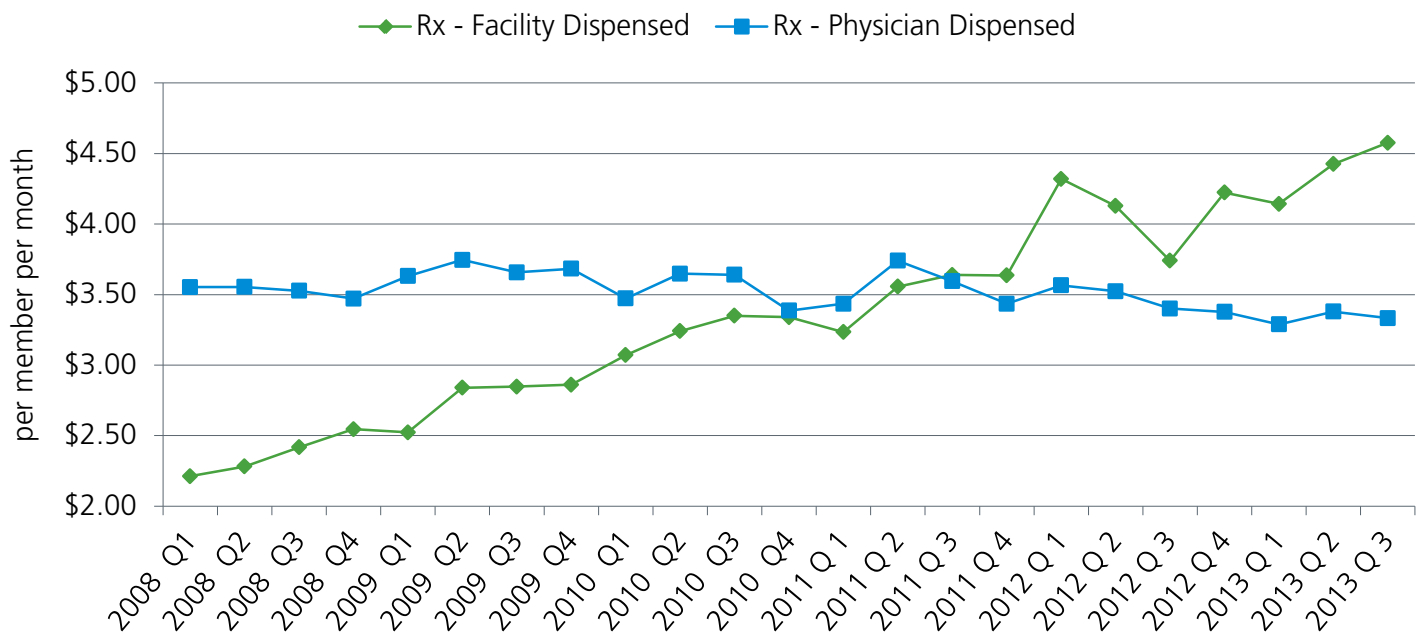
Incentives to prescribe high-cost and high-margin drugs. Under fee-for-service reimbursement approaches, providers can earn more by prescribing high-cost drugs rather than low-cost alternatives. Payers typically pay a percentage of an indexed price; the costlier the drug, the higher the reimbursement. In the Medicare program, the current payment formula of average sale price plus 6 percent for Part B drugs may create an incentive for providers to prescribe high-cost drugs.⁴⁴ Another incentive leads some providers to purchase specialty drugs at a discount from manufacturers and then bill payers a “market” rate and keep the difference; this creates an incentive to keep rates high or to prescribe drugs based on their margin. Oncology practices often derive income from “mark-ups” on chemotherapy drugs – that is, the difference between the practices’ acquisition costs of those drugs and the payments that practices receive for the drugs provided to patients. Addressing those misaligned incentives with revised payment approaches can prove challenging.^{45,46}

Use of high-cost sites of care. Because costs for the same treatment may vary widely depending on the site of care (or method of administration), incentives are leading to the use of high-cost settings and contributing to rising costs. For example, providers of physician-administered and other drugs that are injectable can generate revenues from

administering the drugs as well as caring for the patient, rather than having the patient take those drugs at home. In some cases, that shift in care setting can lead to higher costs. Most notably, there has been a recent shift from administration of injectable specialty drugs in physician offices to hospital outpatient departments, where prices for drugs and administration are about 200 percent higher.⁴⁷ Hospitals frequently charge more for specialty cancer drugs than do physician oncology offices (in some cases 50 percent to 150 percent more).⁴⁸ Ongoing concerns also relate to drugs that treat inflammatory bowel disease and multiple sclerosis; some research, however, questions the extent of those shifts.⁴⁹ Certain hospitals also can purchase specialty drugs at discounts, such as the 20 percent to 50 percent available to some hospitals under a federal program (known as 340B), further increasing their opportunity for profit and incentives to provide drugs in facility settings.⁵⁰ UnitedHealthcare’s data shows that the average mark-up for hospitals is substantially more than that for physicians.⁵¹

The impact of this trend for injectable cancer drugs provided under the medical benefit is shown in Figure 5. While the divergence in per-member-per-month costs shows a shift in care setting, it also reflects contracting dynamics holding down growth in physician costs.

Figure 5; UnitedHealthcare spending by site of service for injectable cancer drugs, 2008-2013



Source: UnitedHealth Group, 2014. Commercial data includes fully-insured membership.

Barriers and limited tools for managing utilization

Traditional pharmacy programs help encourage appropriate utilization of drugs, ensure they are safe for patients, and keep drug costs affordable. Tools include prior authorization, step therapy (starting treatment with a generic or first line treatment), copayment/coinsurance tiers (incentivizing the use of preferred or generic drugs by placing drugs on different levels with progressively higher copayments or coinsurance), and directed networks to manage the use of expensive drugs. It is more difficult to manage specialty drugs using those traditional tools and existing approaches show mixed results.

Private payers and the Medicare program rely on cost sharing tiers to provide incentives for appropriate utilization; in most Medicare Part D plans, beneficiaries have to pay 25 percent to 33 percent of the cost of specialty drugs. Employer-based plans are following suit, with almost a quarter of plans using specialty tiers.⁵² (State Medicaid programs, however, have very limited options to use those tools.) While effective for some drugs, high copayments or coinsurance for certain specialty drugs can deter patients from using the medications they need. A recent study of patients treated for multiple sclerosis found that a 10 percent increase in patient cost sharing resulted in a 9 percent decline in adherence for patients with coinsurance.⁵³ Yet, although manufacturer or provider programs often provide assistance with copayments or coinsurance (with, for example, coupons), those approaches may not ensure specialty drugs are used only when appropriate.

Furthermore, it can be harder to manage utilization under the medical benefit than under the pharmacy benefit. For example, common tools such as prior authorization are more challenging under the medical benefit because of differences in medical and pharmacy claims processing and the level of detail in each system. For example, prior authorization occurs for less than 5 percent of cases.⁵⁴

Government program rules provide barriers to effective management as well. The Medicare Part D program and many state Medicaid programs generally do not encourage effective formulary and network strategies. For example, at OptumRx, 85 percent of specialty medications are dispensed through specialty pharmacies for commercial plan members; by contrast, in Medicare Part D only 15 percent of medications are dispensed through that managed approach.

Treatment complexity, adherence, and quality of care

Patients often struggle with the demands of specialty drugs and do not get the coordinated care they need to effectively address their overall clinical condition. Complex regimens, complications of use and side effects may lead patients to prematurely discontinue their medications or to skip or miscalculate doses. A recent study found that 20 percent of patients took inappropriately high dosages while 13 percent were ‘under-adherent’ with self-administered drugs.⁵⁵ Drug treatments affect medical outcomes and total costs of care and medical factors may influence the effectiveness of specialty therapies. Pharmacy and medical records are often not integrated and accessible to all members of the care team, resulting in an incomplete assessment of the patient’s needs and an incomplete picture of the patient’s condition and response to the specialty drug. Further fragmentation arises when patients use specialty drugs under both the medical and pharmacy regimes. For example, a patient may shift from an intravenous (IV) drug administered by a physician (covered under the patient’s medical benefit) to patient self-administration or oral medications (covered under the pharmacy benefit).

Gaps in data on treatment and utilization

Proper care coordination, sensible payment models, and efforts to improve care all require reliable data about specialty pharmacy utilization and cost. While PBMs can provide spending and utilization data for specialty drugs covered by the pharmacy benefit, those not integrated with a payer do not have the same access to information about the nature of specialty drug spending under the medical benefit and have difficulty tracking spending and utilization data.⁵⁶ Drugs covered under the medical benefit are classified via a different set of identifiers that provides less information than codes used under the pharmacy benefit. Even less information is available for drugs provided in outpatient settings where medical claims systems may combine drug information with other services.⁵⁷ Problems with coding under the medical benefit include multiple drugs sharing the same code, the use of temporary or “miscellaneous” codes for new drugs, and the lack of full information about the amount of a drug used in one instance of care.

Need for more evidence

Evidence-based guidelines and medication therapies are generally well-established for the initial treatment of conditions such as rheumatoid arthritis, hepatitis C and multiple sclerosis. For ongoing treatment and other conditions, providers have less evidence to guide them in selecting medications, treatment duration, and dosage for

their patients. The rapid emergence of new treatments further compounds this challenge. Even less information is available on what constitutes optimal treatment as a disease progresses or as patient responsiveness to the treatments declines over time. As a result, patients may continue to take complex medications that are minimally or no longer effective, incurring the costs of these drugs as well as enduring their side effects.

Meeting the challenge of specialty drugs

Continued innovation will lead to expanded treatment options and improved quality of life for patients with complex conditions. Improved outcomes and lower costs will require proper alignment of payments and incentives, care delivery with effective clinical management and information, and the engagement of providers and consumers in decisions about care.

Solution: Fair and affordable prices for specialty drugs

Currently in the United States, limited tools exist to ensure fair and affordable pricing for specialty drugs. The development of biosimilar drugs, also called follow-on biologics, has the potential to alter the specialty drug marketplace as they might cost 20 percent to 25 percent less than the original biological product.^{58,59} While an approval pathway for such drugs exists, the federal government has not approved any biosimilar or otherwise “interchangeable” drugs to date under that pathway (though a small number of biosimilar drugs have been approved previously).⁶⁰ The future for biosimilar drugs remains uncertain and regulatory barriers may impede their market entry. Additionally, researchers question manufacturers’ ability both to reproduce all aspects of biological processes and to generate real savings.⁶¹ If such drugs do reach the market, it would be necessary to conduct a thorough evaluation of their effectiveness.

Better provider contracting approaches also can unleash efficiencies and help guide patients to higher quality providers. Many health plans, for example, contract with preferred infusion networks or physician groups for cancer treatment and with preferred dispensing pharmacies.

A more fundamental question is whether the high price of a drug is warranted given its effectiveness. Answering this “value” question will involve manufacturers’ willingness to be transparent about how they establish prices and payers, providers, and manufacturers collaborating to develop a true understanding of the relationship between a given specialty drug’s effectiveness and its cost.⁶² With improved information, those stakeholders would be better positioned to negotiate and to develop outcomes-based approaches to

contracting. Advances in comparative effectiveness research may help to inform those discussions.

Solution: Changing market incentives

Changes in payment policy may remove incentives for inappropriate use of high-cost drugs. For example, payers might switch to a payment approach that reimburses drug acquisition costs and provides an additional flat fee to providers (such as one that varies by therapeutic class), rather than a fee that is based on a percentage of the drug’s cost.⁶³ Payers also might provide a financial incentive to providers who use an equally effective and more cost-effective therapy, where one exists.

Reimbursement methods that are neutral concerning the route of administration could encourage the use of the most appropriate and efficient care. For example, in some cases self-administered injectable drugs are less costly and as effective as physician-administered infusion drugs; in contrast, some new oral drugs are substantially more expensive but as effective as their physician-administered counterparts. Payment approaches that reflect the merit of the therapy over the route of administration might help to encourage appropriate use and could include incentives for patients. Some estimates suggest there also are substantial savings opportunities available from guiding patients from hospital outpatient infusion of a drug for rheumatoid arthritis to a physician office, infusion center or a patient’s home.⁶⁴

Bundled payments that include the total cost of care are another approach. For example, the Medicare program pays for treatment of end-stage renal disease with payment bundles that include both pharmacy and medical benefit drugs. Good utilization and cost data are necessary to develop appropriate reimbursement rates for that type of approach.

Responding to substantial variation in total costs of treatment for cancer, UnitedHealthcare developed a pilot program with five medical oncology groups to test a new payment model for cancer care that focuses on best treatment practices and health outcomes. This model uses

an episode-based payment that reflects expected margins for chemotherapy drugs over a standard treatment regimen chosen by the oncology group for a specific condition. Under the pilot, practices receive payment for the cost they incur when buying oncology drugs, so the physician will not gain or lose financially depending on their choice of drug. The pilot separates the oncologist's revenue from drug sales and higher reimbursement is no longer tied to more intensive chemotherapy. The pilot also examined patient survival rates and hospitalizations for complications and encouraged the development of comparative data. UnitedHealthcare will publish results from this pilot in the coming year.

Solution: Encouraging appropriate utilization

Targeted use of traditional tools such as prior authorization can help improve utilization management for specialty pharmacy drugs. Approaches such as targeted step therapy programs can help patients trying different specialty drug options to do so under close consultation with their physicians and care team, with access to specialty consultations. Programs that direct patients to preferred products and disallow coupons when clinically appropriate alternatives are available also are effective in reducing costs while maintaining clinical quality and medication adherence. For drugs with many available options in both the medical and pharmacy areas, patients could benefit from incentives

to choose effective but lower cost care settings, (such as self-injectable versus infusion.)

Other approaches may be particularly effective for specialty drugs. Some prevent waste using better management tools that ensure proper dosage. For example, having an initial prescription for a specialty drug apply for a seven- or 14-day period (as opposed to 30 days) could ensure that patients who experience serious side effects could be provided with alternative drugs as soon as possible with minimal waste. Encouraging the use of diagnostics that can assess the likelihood of a given drug's effectiveness in a specific patient also could reduce misuse and waste. Research shows, for example, that certain patients do not respond to chemotherapy drugs because their cancer does not possess the target gene for the drug. Increasingly, the FDA is requiring that certain new drugs have a so-called "companion diagnostic" test as a condition of approval.

Recommended treatment approaches, known as *clinical pathways*, for certain disease states also offer an opportunity to better manage care by engaging providers more closely in managing the use of drugs in ways that are connected to the best clinical evidence. One health plan reduced costs of care by 15 percent (through lower inpatient hospital admissions and emergency room visits) by using a program that stressed more appropriate use of chemotherapy.⁶⁵ While these actions may not always impact costs, they do make spending more

Case Study; OptumRx specialty pharmacy programs and synchronized medical and pharmacy services

OptumRx, one of the nation's largest PBMs, provides specialty pharmacy services, including clinical management and tailored programs for patients taking 400 medications across 25 therapeutic categories and specialty disease states, including cancer, rheumatoid arthritis, and multiple sclerosis. A central feature of those programs is that patients are assigned to a clinical pharmacist or a registered nurse with specialty pharmacy training for consistent care with one-on-one consultations, ongoing case management, and financial assistance program referrals. Additionally, programs provide patients with education and counseling, guidance on symptoms related to their medical conditions and drug side effects, proactive intervention for medication non-adherence, and prospective refill reminder and follow-up calls. OptumRx's specialty pharmacy can provide enhanced clinical management of patients taking specialty pharmacy drugs and synchronize all of the services needed by a given patient. The goal is to improve patient outcomes and reduce total costs of care. A consolidated view across pharmacy and medical services that integrates data and analytics provides insights into spending and utilization trends, including the roughly half of specialty drug spending provided under the medical benefit. Those insights increase the specialty pharmacy's ability to engage patients and manage patient care more broadly. This synchronization model embeds specialty pharmacists in clinical care teams to help coordinate medical and behavioral benefit services and supports.

predictable for health plans and patients. Reaching out to physicians with comprehensive guidance and advances in new drug development also can help to ensure the appropriateness of specific treatments.

Entities that support those targeted utilization approaches include specialty pharmacies, which are staffed with pharmacists trained in the administration and use of specialty drugs. Specialty pharmacies can review patient case histories and use the information learned to adapt existing guidelines to educate patients and improve prescribing practices.

Solution: Improving medication adherence and care coordination

Clinical management services that include individualized patient education and support and disease-specific therapy programs are essential to improving medication adherence and care coordination. Before beginning treatment with specialty drugs, patients need to understand their condition, expected treatment side effects, expected long-term outcomes, and the costs they will face. It also helps patients to understand complex clinical regimens including the importance of the order and the timing of taking the drugs to maximize treatment effectiveness. Clinical management services also can guide patients to the most appropriate site of care for their condition: whether receiving care in an outpatient hospital care setting warrants its cost and the risk of illness or infection, for example, or ensuring that the proper equipment is in place for home care. Side effects can be anticipated and medications to alleviate these can be ordered in advance.

Benefits of the specialty pharmacy approach. Greater use of specialty pharmacies can increase patient access to those clinical management services (see Case Study on previous page); those pharmacies have drug compliance rates that are nearly 10 percent higher than those seen in the standard retail pharmacy sector. For example, research published in peer-reviewed journals on OptumRx specialty programs shows that the clinical supports offered by these programs are associated with improved adherence for oral oncology and renal transplant drugs and injectable multiple sclerosis and rheumatoid arthritis therapies, greater reductions in total costs of care (13 percent for oral oncology and renal transplants), and with improved outcomes such as a reduction in multiple sclerosis relapses. (See Appendix A for summaries of the research.)

Another example of the value of specialty pharmacy comes from the Pennsylvania Medicaid program. The program combines payment negotiation, care coordination, and expanded clinical management to achieve cost savings. Nurses administer specialty drugs when prescribed by the physician, staff operates a 24/7 call center for beneficiaries, and the program provides case management services. The state reports saving 21 percent on overall per-member-per-month expenditures for members who used specialty drugs.⁶⁶

Value of integration of medical and pharmacy benefits.

Combining specialty pharmacy and health plan capabilities holds great promise for improving care coordination and adherence. A recent study of an integrated medical and pharmacy program found that cost reductions are possible, despite the fact that half the cost of care is for the specialty drugs themselves.⁶⁷ This model uses intensive clinical support and care management, guided by information from the patient's medical and pharmacy records and patient self-reports about the effects of treatment, to manage an individual patient's care. Information from this range of sources then feeds into electronic health records available to all members of the care team. Clinicians then have access to up-to-date information on companion diagnostics or laboratory results that may affect dosages of specialty medications.

The model supports patients' and clinicians' joint review of the patients' experiences with the medications, so clinicians can make required adjustments to treatment and refer patients to any other services that might be needed (for example, patients who administer their own specialty drugs may need additional social support). Counseling can help patients understand the need to stay on medications until their condition is fully resolved, despite fluctuations in symptoms. Integrated programs also can support palliative and hospice care by working with the patient, family and care team to provide both appropriate medications, and other needed services.

Patient and provider engagement. For patients and their families, greater engagement in self-management of specialty medications will help to improve their outcomes. It is vital for patients to share with their care providers experiences they have with their drugs and the broader impact on their quality of life so that adjustments in therapies may be made before problems occur. Oral chemotherapies, in particular, have the potential for serious toxicities so patients benefit from reporting their symptoms

and progress to their providers. Self-administration of specialty medications can be a benefit for patients (e.g., less travel, more privacy) but also can be a burden because that approach may yield less face-to-face support. Education and social support programs should assist patients and their families in those activities and in broader efforts to maintain healthy lifestyles during treatment. The ability of care providers to maintain “high-touch” engagement with patients on specialty drug regimens is an important way to motivate adherence. Use of multi-specialty care teams including advanced practice nurses will be beneficial, as will use of specialty pharmacists.

Solution: Improving information and evidence to support high-quality care

Any of the initiatives described in this paper to improve the management of specialty drugs requires good data about which drugs are used, in what manner, and for what amount of time they are provided, as well as accurate information about a drug’s impact on the patients who receive them. Better coding systems are needed to capture the data about specialty drug utilization and stronger links between coding, claims payment, and medical records systems will help make the connection between drug utilization and resulting patient outcomes. This might involve monitoring use when there is no clear code or when spending exceeds a certain threshold.

Strong incentives for physicians to provide more detailed information about response to treatment and outcomes for payers and consumers could help to increase the knowledge base. For example, in the treatment of cancer and of HIV, providers collaborate with medical specialty societies and other key stakeholders to create a database or registry of treatment outcomes. Providers and patients furnish data on changes in treatment over time, including patient-level information about symptoms and side effects and population-level data that helps physicians identify comparable patients and the effect of the treatment they received. Registries such as these have proven invaluable in the care of patients with long-term illnesses that require complex treatment and medications—exactly the types of patients most likely to be using specialty drugs. Payers and providers (such as accountable care organizations) could require participation in such registries or databases for their beneficiaries or members receiving specialty drugs.

Data are only a start; research is needed to create evidence that then can guide long-term treatment. Currently, measures exist that can be used to assess short-term benefits of specialty drugs, such as symptom relief or the resumption of regular activities. Further work is needed on approaches for measuring long-term outcomes, such as increased survival rates or a reduction or absence of disease progression. Greater investment in comparative effectiveness research can help add to the evidence base and help patients sort through treatment options. (See Case Study below.)

Case Study; Optum Labs

Optum Labs is an open center for collaborative research and innovation, providing unique data and analytic resources that enable stakeholders from across the health care system to drive advances that will lead to improved patient care. Researchers in Optum Labs come from partners spanning the health care system, all of whom are interested in working collaboratively with the largest collection of de-identified, linked administrative claims, and electronic health record data, using state-of-the-art analytic tools.

This unprecedented linked data set and tools to explore the data allows comprehensive insights on very large populations and is ideal for rapidly expanding the evidence base for many areas of comparative effectiveness research. In the area of specialty pharmacy, the Optum Labs data serve as a unique resource to evaluate the effectiveness of specialty drugs, many of which are created to treat rare, complex diseases, and to benefit from the larger amount of data available to analyze outcomes for different patient populations. Researchers also benefit from the ability to study the natural history of complex chronic diseases and evaluate the impact of changing drug treatment regimens as the patient’s disease progresses or recedes. This in turn enables researchers to identify which treatment, works best to improve outcomes for individual patients, the optimal timing and length of treatment, and whether long-term complications can be reduced or eliminated. Researchers then translate the findings into improved health for the population.

Conclusion

Scientific advances and innovation in specialty pharmacy will accelerate in the coming years, adding to the arsenal of beneficial cures and treatments. Continued growth in the prevalence of chronic disease also will affect this trajectory. To ensure the benefits of these new treatments are fully realized, there are a number of steps that can now be taken. Ensuring patients get the right drug in the most appropriate setting will depend on revised payment models; evolution of accountable care organizations may help spur improved approaches. Greater use of care coordination and adherence programs - of the kind provided by specialty pharmacies - shows particular promise in improving outcomes and reducing costs. New approaches to payment design, clinical management, and data analytics will help. And a more integrated view into pharmacy and medical benefits can enhance those efforts.

Appendix A: Select peer-reviewed OptumRx research on specialty pharmacy programs

The following peer-reviewed journal articles show the value of specialty pharmacy programs in addressing the needs of patients with complex conditions and treatments:

Injectable drugs for multiple sclerosis patients. One study examined the impact of an OptumRx medication therapy management program that teaches patients with multiple sclerosis how to use injectable specialty drugs, provides advice on managing side effects, and educates patients about potential complications through consultation with a nurse or pharmacist. **The study found that the patients in the program demonstrated greater adherence to injected medications and treatment schedules than patients who obtained specialty medications without the program either through a retail pharmacy or specialty pharmacy (92 percent compared to 86 percent and 90 percent respectively).**

The relapse rate for the intervention group decreased by 4.7 percentage points (from 14 percent to 9.3 percent). The program saved an estimated \$13,026 per relapse. (Stockl, KM, Shin, JS, Gong, S., Harada, ASM, Solow, BK, Lew, HC, *Improving patient self-management of multiple-sclerosis through a disease therapy management program, AJMC, 6(2), Feb. 2010*)

Injectable drugs for rheumatoid arthritis. OptumRx initiated a specialty pharmacy program for patients with rheumatoid arthritis that focused on adherence to injectable drugs and compared the results to results from patients who received their medications from a community pharmacy. Patients in the specialty pharmacy program received education, support, and assistance in patient management of symptoms from a pharmacist or nurse and demonstrated significantly higher rates of medication adherence compared to community pharmacy patients. (Stockl, KM, Shin, JS, Hew, HC, Zakharyan, A., Harada, ASM, Solow, BK, Curtis, BS. *Outcomes of a rheumatoid arthritis disease therapy management program focusing on medication adherence. J. of Managed Care Pharmacy, Oct. 2010, 16(8), 593-604*)

Oral drugs for renal transplant patients. A retrospective claims analysis compared costs and outcomes for patients who filled specialty oral prescriptions for anti-rejection drugs through an OptumRx specialty pharmacy program, which provided personalized 24-hour pharmacist/nurse consultations for patient education and support, monthly check-ins and refill calls, management of expected adverse effects, and referrals to clinical specialists for care support to costs and outcomes for patients who received standard pharmaceutical care (including a base level of communication and follow-up for missed dosages) at a retail pharmacy. Fewer patients experienced gaps or stopped taking medications in the specialty program, a common problem as transplant patients often stop taking their medications because of side effects, confusion or forgetfulness about the timing of drugs. **After one year, total medical and pharmacy costs for the specialty program participants were 13 percent lower than for those in the comparison group.** The specialty pharmacy patients also demonstrated a 30 percent greater reduction in transplant-related medical cost such as inpatient and outpatient surgery, drugs, follow-up visits, and dialysis resulting from organ rejection compared to those patients not in the program. (Tschida, SJ, Aslam, S, Lai, LS, Khan TT, Shrank, WH, Bhattarai, GR, Sahli, BD, *Managing Specialty Medication Services through a Specialty Pharmacy Program: The Case of Oral Renal Transplant Immunosuppressant Medications, Journal of Managed Care Pharmacy, January/February 2013, Vol. 19, No. 1.*)

Oral drugs for cancer. Another study analyzed the impact of an OptumRx specialty pharmacy program on costs and outcomes for patients who take new targeted oral oncology medications, which are both expensive and often toxic. Patients in the study received individualized care from trained nurses and pharmacists; the study compared their experience to patients who purchased oral medications from a retail pharmacy without such supports. Pharmacy costs increased for both groups due to the higher costs of

the oral chemotherapy drugs; however, **total costs after one year of the study were 13 percent lower in the specialty pharmacy group than in the comparison group** (about \$84,000 in the specialty program versus \$97,000). Differentiating factors included greater medication adherence, fewer outpatient visits, and lower costs in the specialty pharmacy group (about 40 percent lower, due in

part to avoided intravenous chemotherapy treatment and adverse events). (Tschida, SJ, Aslam, S, Lai, LS, Khan TT, Shrank, WH, Bhattarai, GR, Montague-Clouse, JC, Sahli, BD, Newcomer, LN, *Outcomes of a Specialty Pharmacy Program for Oral Oncology Medications*, *The American Journal of Pharmacy Benefits*, 2012; 4(4):165-174)

Appendix B: Additional data charts

Figure 6; Distribution of spending for specialty drugs by type of benefit and disease state, UnitedHealthcare Medicaid health plans, 2012

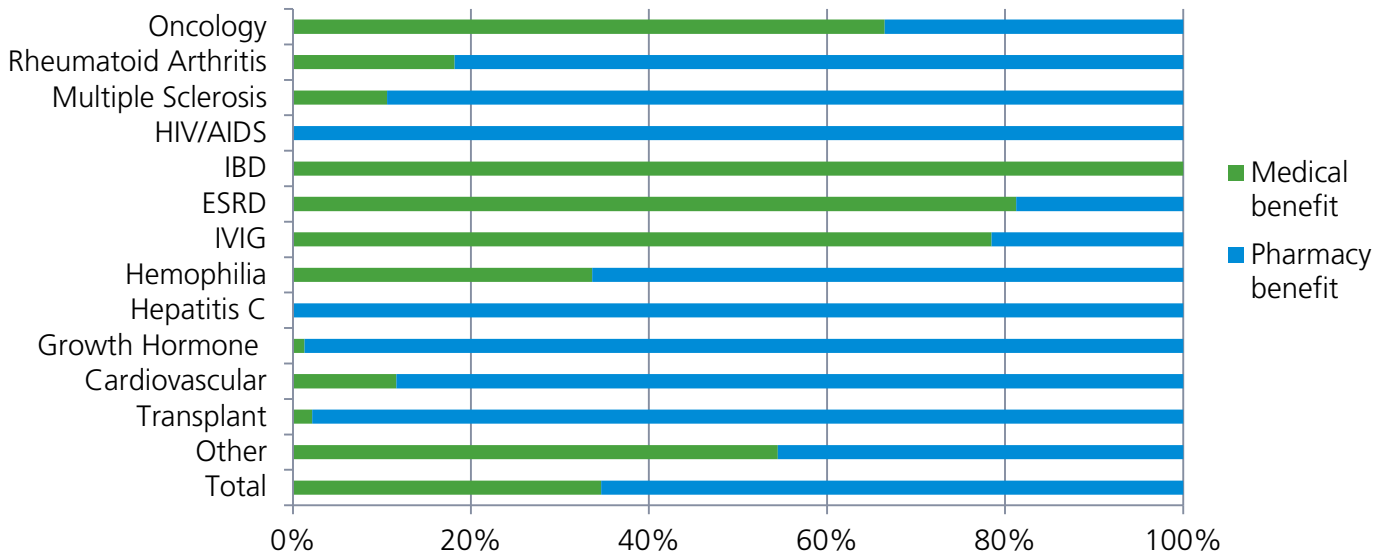
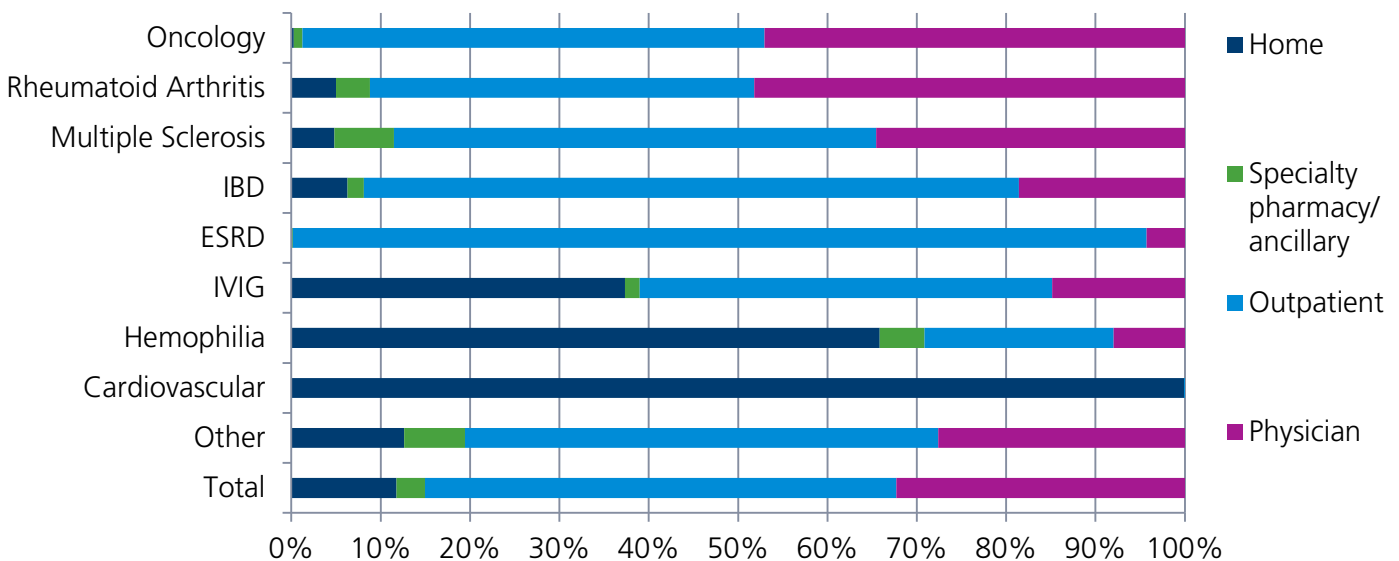


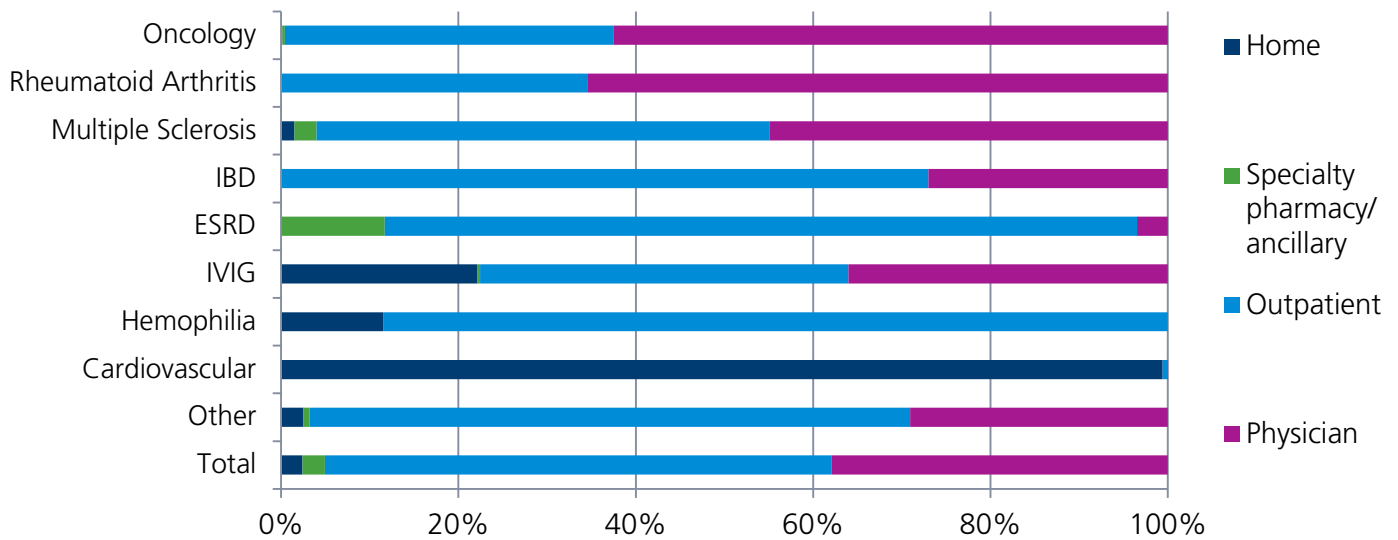
Figure 7; Distribution of spending for specialty drugs by setting of care and disease state, medical benefit, UnitedHealthcare Medicaid health plans, 2012



Source: UnitedHealth Group, 2014

Notes: IBD = inflammatory bowel disease, ESRD = end-stage renal disease, IVIG = intravenous immunoglobulin

Figure 8; Distribution of spending for specialty drugs by setting of care and disease state, medical benefit, UnitedHealthcare Medicare Advantage plans, 2012



Source: UnitedHealth Group, 2014

Notes: IBD = inflammatory bowel disease, ESRD = end-stage renal disease, IVIG = intravenous immunoglobulin

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