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Specialty Pharmaceuticals. Complex new drugs hold great promise for people with chronic and life-threatening conditions. The drugs are also a driver of spending growth.

WHAT'S THE ISSUE?

Specialty pharmaceuticals are a rapidly growing share of total drug expenditures by public and private health plans. These drugs, typically used to treat chronic, serious, or life-threatening conditions, such as cancer, rheumatoid arthritis, growth hormone deficiency, and multiple sclerosis, are often priced much higher than traditional drugs. Total costs can be in the thousands of dollars a month and can exceed \$100,000 a year for some products. There are usually few if any low-cost generic equivalents.

These high costs represent an increasing burden on payers, including governments and employers. Because health plans often require substantial cost sharing for specialty drugs, there is also a large impact on patients who need such medicines. Some are concerned that cost sharing could discourage the use of these drugs, perhaps leading to adverse health outcomes.

Research into specialty drugs is attractive to biopharmaceutical companies from both a medical innovation and business perspective. The ability for the market to sustain the relatively high cost of these products creates substantial incentives for companies to research and develop products that address serious, unmet health needs. Also, in some

cases, companion diagnostic tests are used to identify patient subpopulations in which the specialty product generates a differential response, either negative or positive. As a result, specialty products have stimulated diagnostic research. Given the level of investment, this means that patients and payers can expect continued innovation and research to develop such medicines and companion diagnostics in the future.

Payers are interested in steps they can take to control the contribution that specialty pharmaceuticals make to the growth in premium costs while ensuring that patients can access those drugs that will improve their health and quality and length of life. Biopharmaceutical manufacturers and patient advocates are concerned that restrictions on specialty pharmaceuticals could discourage research and harm patient care. Pharmacists are also concerned that some plans to control the use and cost of specialty pharmaceuticals will limit their opportunities to serve patients.

WHAT'S THE BACKGROUND?

Although there is no accepted definition of *specialty pharmaceuticals*, they generally are drugs and biologics (medicines derived from living cells cultured in a laboratory) that are complex to manufacture, can be difficult to administer, may require special patient mon-

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itoring, and sometimes have Food and Drug Administration (FDA)–mandated strategies to control and monitor their use. Increasingly, specialty pharmaceuticals have come to be defined by exceeding a certain threshold cost, such as \$600 per month, that may place such drugs on higher cost-sharing tiers.

From a distribution perspective, specialty pharmaceuticals may require specialized shipping and temperature-controlled storing and handling. Therefore, a class of providers, known as specialty pharmacies, exists to distribute and dispense these products. Such pharmacies can be owned by stand-alone companies, pharmaceutical benefit managers (PBMs), or large pharmacy chains. Specialty pharmacies ship the product to the physician and bill the patient’s insurance company. In contrast, some drug wholesalers also have specialty distribution capabilities to ship specialty products directly to physicians who then buy the product and bill the patient’s insurance company.

As specialty pharmaceuticals can include oral and self-injectable drugs and physician-administered infusions, they may be covered under the medical or pharmaceutical benefits of health plans. Although estimates differ depending on data sources, Express Scripts’ drug trend report claims that in 2010 approximately 47 percent of US specialty drug spending occurred in the medical benefit.

According to IMS Health, in 2012, 65 percent of spending on new drugs (those introduced in the past two years) was on specialty pharmaceuticals. Statistics in this area also may vary substantially based on different sources and different definitions of specialty drugs.

Experts expect that share to continue to rise as specialty pharmaceuticals represent a critical growth segment in drug development. CVS Caremark and Express Scripts, two of the largest PBMs, report that specialty pharmaceuticals are the fastest-growing segment of their drug spending, and by 2019 they expect specialty drugs to represent 50 percent of their plan sponsors’ overall drug spending. In 2013 at least 60 percent of the new drugs expected to be approved for marketing in the United States by the FDA will be specialty pharmaceuticals, according to Express Scripts.

From a patient perspective, new specialty pharmaceuticals represent more-effective ways to treat serious diseases—many of which

currently have few available options. Where such products exist, initiation of treatment is occurring sooner as physicians seek to address chronic, serious, or life-threatening conditions. As a result, costs accruing to the health plan occur earlier and last longer, particularly for products used to treat chronic diseases.

WHAT’S THE DEBATE?

Payers are seeking ways to appropriately manage the growing cost of specialty pharmaceuticals. In many cases, the traditional means of influencing drug pricing, such as tiered formularies and drug rebates, are not available for specialty pharmaceuticals because many do not have close therapeutic alternatives.

COST SHARING: Payers are employing a variety of tools to control cost. One common tool for specialty medicines covered through the outpatient pharmaceutical benefit is to charge high cost sharing in the form of copayments (fixed amounts) or coinsurance (percentage amounts) by placing specialty drugs in their own formulary tier. In Medicare Part D, where the use of a specialty drug tier is common, beneficiaries are typically charged 25–33 percent of the cost of their specialty drugs, although the structure of Part D reduces such costs substantially once beneficiaries reach an out-of-pocket limit. Beyond Part D, the percentage of employer-sponsored plans that use specialty tiers has increased from 14 percent of plans in 2012 to 23 percent in 2013, according to the Kaiser Family Foundation’s 2013 Employer Health Benefits Survey. Starting in 2014 health plans offered under the Affordable Care Act’s (ACA’s) health insurance Marketplaces will also have specialty pharmaceutical tiers. However, the impact of such tiers on patients may be mitigated in part because of the ACA’s out-of-pocket maximums.

Critics of this trend contend that high cost sharing discourages appropriate use of medicines. Studies show that increased cost sharing leads to higher abandonment of prescriptions. This is especially a concern for the serious and chronic conditions that are the target of most specialty pharmaceuticals.

To mitigate the cost to patients and encourage treatment, many specialty pharmaceutical manufacturers offer assistance programs to patients. These programs provide coupons to patients for some, or all, of the amount of cost sharing. In the case of Medicare, where such assistance is not allowed under federal

65%

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law, some manufacturers provide grants to independent nonprofit organizations, which in turn are able to reimburse patients a portion of their share of the costs of medicines.

These discount programs have been criticized by payers and PBMs because they reduce the incentives that are embedded in traditional benefit designs and may lead to overuse. Therefore, even though copayment assistance programs may make a particular product more affordable for an individual patient, some contend that they increase the overall costs to the plan and all members covered under the plan by encouraging the product’s use. Manufacturers and patient advocates defend these discount programs, arguing that the cost-reduction incentives of traditional benefit design should not be applied to specialty products because of the seriousness of the diseases they treat and the small patient populations involved. In addition, by making these high-cost therapies more affordable to patients, coupons and copayment assistance can help reduce nonadherence and its consequences, such as emergency department visits and hospitalizations.

In addition to high cost sharing, some payers are requiring diagnostic testing as a condition of coverage to identify individuals who demonstrate a propensity to respond to a particular specialty product. Similarly, managing the site of service is an important issue as more specialty products can be administered by the patient at home instead of in more expensive sites of care, such as outpatient facilities or the physician office.

MEDICAL BENEFIT VERSUS PHARMACY BENEFIT: Because specialty drugs are often reimbursed under the medical benefit and their costs may be bundled in with other services, payers have less direct influence over pricing. Therefore, payers and PBMs are exploring ways to move specialty products from the medical benefit to the more transparent pharmacy benefit to better influence pricing and gain information on use and outcomes.

Critics of this trend are concerned that this could harm care by reducing the role of physicians in assisting patients in the proper use of their medicines. In many cases, time is of the essence in starting a patient on a specialty drug treatment regimen. As a result, the amount of time and effort required for physicians, office staff, patients, and caregivers to navigate pharmacy benefits’ utilization restrictions can be onerous.

Administering specialty products is often a driver of physician office revenue. Critics contend that new administrative burdens imposed as part of a pharmacy benefit may undermine office practice efficiency and profit.

Distribution channels also offer an area for managing cost. For some products, payers are seeking to limit the type and number of pharmacies that can distribute a specialty pharmaceutical to patients to gain economies of scale and concentrate purchasing power. These limited networks may also be able to improve the ability of health plans to implement care protocols, improve adherence, avoid product waste, and implement FDA-required use plans. These efforts to limit who can distribute specialty pharmaceuticals often clash with the interests of retail pharmacists and physician business models built in part on the revenue from administering specialty drugs.

BIOSIMILARS: The Affordable Care Act directed the FDA to create a new approval pathway to allow the sale of clinically equivalent versions of biologically derived therapies known as biologics. Patient advocates and payers hope that the introduction of these follow-on versions of biologics—known as biosimilars—will force prices down in the same way that generic drugs compete with traditional brand-name drugs. In the United States to date, there have been no medicines approved under this new authority, and many experts believe that even once approved, biosimilars will offer cost savings but not of the magnitude seen with generics. Nevertheless, some are concerned that patients will be switched, with or without their knowledge, to biosimilars, which may not achieve the same results as the original drug. (See a previous Health Policy Brief for additional background on [biosimilars](#), published October 10, 2013.)

COMPARATIVE EFFECTIVENESS: In recent years, the growth in the use of comparative effectiveness review has created another tool to control the increase in specialty pharmaceutical costs. Proponents believe that comparative effectiveness review can provide information that will allow payers to appropriately deny coverage for products that cost more than alternatives but do not provide any additional clinical value.

Opponents question whether such reviews, especially those conducted by payers, can be unbiased and whether these population-based analyses take into account the fact that individual patients’ responses to treatments vary.

(See a previous Health Policy Brief for additional background on [comparative effectiveness review](#), published October 8, 2010.)

Pressure from individual patients and patient advocates and limited resources has restricted the ability of comparative effectiveness review to substantially alter coverage for specialty pharmaceuticals. Such review is not currently allowed in Medicare Part B but may be considered by Medicare Part D drug plans and private health plans. Outside the United States, government payers that are similarly concerned about the high cost of specialty pharmaceuticals have been more aggressive in their use of comparative effectiveness reviews.

The United Kingdom has been particularly wary of providing access to high-cost products without a rigorous demonstration of improved patient outcomes compared to existing, less expensive treatment alternatives. One of the most publicized challenges of a specialty product occurred when the United Kingdom's National Institute for Health and Care Excellence did not recommend bortezomib (Velcade) monotherapy for the treatment of relapsed multiple myeloma to be covered by the National Health Service because of cost concerns. The manufacturer engaged in a negotiation to gain coverage based on actual patient outcomes, effectively offering the National Health Service a rebate if particular outcomes were not achieved. Such negotiations based on outcomes may have a future in the United States. However, because many are very sensitive to suggestions of rationing care, implementation may be challenging.

WHAT'S NEXT?

Given that the growth of the specialty pharmaceutical market is expected to continue for

many years, discussions about the right balance between cost containment and patient access will continue. These discussions will play out in private forums among payers and specialty pharmaceutical manufacturers. They will also spill into public debates over the role of government in encouraging policies that will impact specialty pharmaceutical access and cost.

The Patient-Centered Outcomes Research Institute, created by the Affordable Care Act, could be a substantial new source of funding for research on the effectiveness of specialty pharmaceuticals, although such studies are often costly and difficult. In addition, the Independent Payment Advisory Board (IPAB), also created by the ACA, has the potential to authorize changes in the Medicare program to reduce payments for specialty medicines. However, as of now, the law has not triggered IPAB's authority to act.

Attention will focus on the ACA's Marketplaces and the access to specialty pharmaceuticals in Marketplace health plans. Debates will also continue on questions that may impact the uptake of biosimilar products—once the FDA begins to approve them. Already state legislatures have begun to consider and enact laws to regulate biosimilars.

Finally, questions about the ability of specialty pharmaceutical manufacturers to assist patients in paying cost sharing through the use of coupons and other means will continue in private and public forums. ■

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