National Business Group on Health®

POLICY **Brief**

JANUARY 2017

Policy Recommendations to Promote Sustainable, Affordable Pricing for Specialty Pharmaceuticals

A *National Business Group on Health*® Publication

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Introduction

Expenditures for specialty drugs are growing faster than any other component of healthcare spend,[1] well above the rate of overall health care inflation[2] and far outpacing that of general inflation, overall growth in the economy and wages.[3] Moreover, the number of drug approvals, spending and utilization for specialty medicines are projected to overtake traditional pharmaceuticals over the next several years. These trends add to the growing sense of urgency for large employers, who are continuing to strategize on how best to manage growing pharmacy expenditures, and for employees, who are paying more out-of-pocket for these medications. They are not alone. Insurers, governments, patients and others are also concerned about growing expenditures for specialty medications.

This policy brief describes the challenges large employers face in managing high-priced specialty drugs, examines ways to better manage them and offers public policy recommendations that would create a more favorable environment for financial sustainability and affordability of these medications.

Specifically, this brief explores the dynamics of the specialty pharmacy market—including the growth in specialty pharmaceutical development; price and price inflation; increases in utilization for an expanding list of conditions; and the impact of vertical consolidation on specialty drug prices. Next, it identifies areas of opportunity for employers to better manage specialty drugs, followed by a look at public policy barriers to better pricing. The document concludes with public policy recommendations for more sustainable, affordable pricing.

What is a Specialty Drug?

Characteristics often include:

- Expensive (at least $600/month for Part D)
- Manufactured using living organisms (biologicals)
- Difficult to administer; may be injected or infused
- Prescribed by specialist physicians
- May require complex patient follow-up, monitoring
- Used to treat serious conditions for which few or no alternative therapies are available
- Administered through specialty pharmacies
- Require special handling (temperature control)

While reducing or removing policy obstacles to better pricing will be a major step forward, a large part of the solution rests with manufacturers. Ultimately, they will need to address the growing concerns about pricing and adopt pricing models that respond to and better reflect those concerns. Viable solutions will also require collaboration among patients, payers (including employers), manufacturers, policy makers and providers. Failure to take action threatens to jeopardize both the ability of people to afford vital specialty medicines and the ability of insurers, employers, and governments to pay for them.

Reasons Behind the Spending Increases

Rapid Growth in Specialty Pharmaceuticals and Spending

The recent rise in spending for specialty pharmaceuticals is due to a number of factors. Year-over-year drug approvals by the U.S. Food and Drug Administration (FDA) have been on the rise, and over the past five years, more of these approvals have been for specialty medicines than for traditional pharmaceuticals. In fact, 2014 was a landmark year for drug approvals overall: An 18-year high of 51 new therapies were approved.[4] Twenty-seven approvals were considered specialty drugs. In 2015, specialty drug products accounted for approximately half of the 45 new drugs and biologics approved by the FDA.[5] And, even while 2016 closed with a notably low number of overall drug approvals, with 22 novel approvals, nine of those were for rare diseases, six were for various types of cancer, two were for types of Hepatitis C, one was for side effects associated with Parkinson's Disease, and one was for a rare liver disease. Thus, a rough analysis suggests that at least 19 of the 2016 approvals could be considered specialty drugs.

The growth in approvals for specialty drugs, along with an increase in the number in the pipeline, can be attributed largely to a growing understanding of disease accompanied by technological advances needed to develop specialty medications. Thus, a class of drugs once thought of only in the context of rare and life-threatening diseases is today utilized for an expanded population and to treat more common conditions.

As more specialty drugs are hitting the market, with broader indications applicable to larger patient populations, expenditures for these drugs have also gone up. In fact, total spending on medicines in the U.S. reached $310 billion in 2015 on an estimated net price basis[i], up 8.5% from the previous year, according to the IMS Institute for Healthcare Informatics. Specialty drug spending accounted for $121 billion on a net price basis, up more than 15% from 2014.[6]

Overall, 2015 saw a 21.5% spending increase for specialty medicines, reaching $150.8 billion on an invoice price basis.[ii]

Put another way, the estimated number of Americans who experienced annual drug expenses greater than $50,000 increased 63% in 2014, from 352,000 people to 576,000.[7] Many of these patients take multiple drugs, and 92% use high-priced specialty drugs.[7]

Though employers are stepping up utilization management for specialty medications,[8] they haven't been able to do so quickly enough to mitigate the rapid growth in drug expenditures.

Growth in Specialty Drug Development and Spending Expected to Continue

Investment in the development of specialty medicines has ballooned over the past decade, and the true estimates of what to expect in the future are largely unknown.[9] But it is possible to make some predictions based on current trends.

As a starting point,[9] for the first time in history, more than 50% of the drugs in development are considered specialty drugs. Because of this focus on development, specialty drugs are expected to make up 50% of overall drug spending by

i According to IMS, "net-price spending" is an estimate of the amount received by pharmaceutical manufacturers and therefore reflects rebates, off-invoice discounts and other price concessions made by manufacturers to distributors, health plans and intermediaries.

ii According to IMS, "spending on medicines" and "invoice-price spending" refer to the amounts paid to distributors by their pharmacy or hospital customers. It does not relate directly to either the out-of-pocket costs paid by a patient or the amount health plans pay for the medicines, and does not include mark-ups and additional costs associated with dispensing or other services associated with medicines reaching patients.

2018.[10] By 2020, nine of the ten best-selling drugs by revenue are projected to be specialty drugs, compared with three drugs in 2010 and only seven in 2014.[11]

It's Not Just the Initial Price of New Drugs – It's also the Price Increases for Existing Drugs

Development trends and launch pricing are important, but equally important is the unprecedented and consistent inflation in the unit prices of specialty drugs that have been on the market for a while. For example, the average annual cost of cancer drugs increased from roughly $10,000 before the year 2000 to over $100,000 by 2012, according to a recent study in Mayo Clinic Proceedings.[12]

Over the last year, a handful of drug price increases dominated the media and caught the public's eye, but price inflation is not limited to select outliers. In July 2016, the Wall Street Journal reviewed corporate filings and conference-call transcripts of the 20 largest branded manufacturers and found that more than two-thirds had attributed their sales increases in the first quarter at least in part to raising prices.[13] This practice was observed for both traditional pharmaceutical products nearing patent expiration, as well as for specialty products with already high price tags.

Overall, nearly one-third of branded drugs experienced annual price increases of 20% in 2015.[14] An Express Scripts analysis of overall trend and unit price increases for specialty drugs found that all of the top 10 specialty therapy classes increased in spend, and all had increases in unit prices of medications.[14] And within the top ten, three specialty therapy classes – inflammatory conditions, multiple sclerosis and oncology – accounted for 56.3% of all expenditures (see Table 1).[14]

Table 1: Price Increases of Specialty Drugs by Therapy Class

			TREND		
RANK	THERAPY CLASS	PMPY SPEND*	UTILIZATION	UNIT COST	TOTAL
1	Inflammatory conditions	$89.10	10.3%	14.7%	25.0%
2	Multiple sclerosis	$53.31	3.5%	6.2%	9.7%
3	Onocology	$49.62	9.3%	14.4%	23.7%
4	Hepatitis C	$38.44	-2.2%	9 2%	7.0%
5	HIV	$31.53	4.6%	12.0%	16.6%
6	Growth deficiency	$7.12	2.8%	2.8%	5.6%
7	Cystic fibrosis	$6.64	12.5%	40.9%	53.4%
8	Pulmonary hypertension	$5.85	13.4%	4.8%	18.1%
9	Hemphilia	$5.79	4.9%	15.4%	20.4%
10	Sleep disorders	$4.57	5.5%	18.5%	24.1%
	TOTAL SPECIALTY	**$341.21**	**6.8%**	**11.0%**	**17.8%**

Source: Express Scripts[14]

*Per Member Per Year

Utilization of Specialty Drugs Has Also Increased

Compounding the problem of high per unit prices and the dramatic growth in spending, utilization of specialty drugs has swelled in recent years, contributing to higher spending. As mentioned earlier, historically, specialty drugs were used to treat only a small subset of diseases, but with recent advances in technology and a greater research and development focus on more common conditions, physicians now prescribe these drugs for a wide range of conditions. In 1990, only 10 specialty drugs were on the market, but today there are more than 300, which has had the effect of drastically expanding the number of patients who take these drugs.[7]

For example, in 2014, specialty utilization rose by 5.8% because of increased use of existing drugs and the introduction of new pharmaceuticals.[7] Just a year later, in 2015, utilization rose almost 7%, and unit cost increased by 11.0%.[14] Furthermore, seven of the top 10 therapies had increases in utilization.[14]

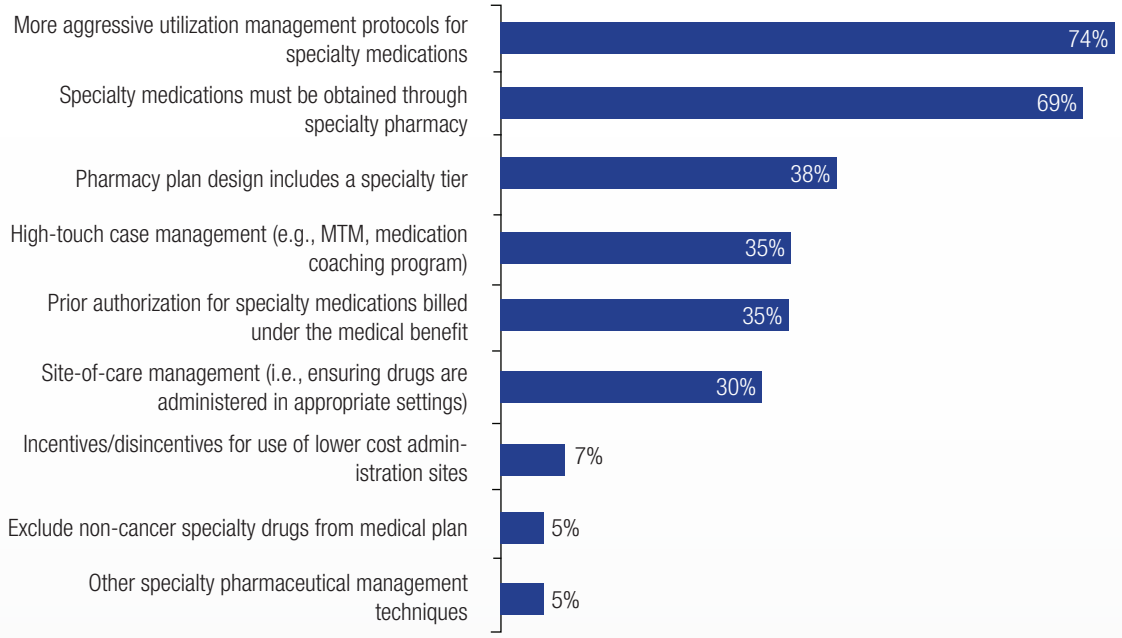
While we cannot pinpoint the potential growth in utilization in the coming years, we can make predictions about spending. Estimates suggest that spending is forecast to increase around 17% annually between 2016 and 2018.[14] This growth is expected to occur as existing specialty drugs gain approval for other indications and are prescribed more often, coupled with FDA approval of new therapies. All of these factors will continue to drive increased utilization.

Further, projections may not adequately account for several key factors that could have a significant impact on growth: what drugs the FDA will approve, how widely those drugs will be utilized, or how effective those drugs will be in terms of delivering value. These factors will be particularly important when less expensive alternatives of comparable safety, efficacy and quality are available.

What Can Employers Do?

As concerns about specialty drug spending grow, making it increasingly difficult for employers and other payers to anticipate and plan for the continued rise in expenditures, they have implemented changes in benefit design intended to preserve high-quality, high-value care while also addressing their increasing cost challenges. Tactics such as site-of-care management, specialty tiers, pharmacy price transparency programs, and requiring the use of specialty pharmacies have all become significantly more widespread since last year.[15] These and other cost-containment strategies are shown in Figure 1.

Figure 1: Large Employers' Specialty Pharmacy Benefit Management Techniques[16]



With the exorbitantly high prices of specialty drugs, ensuring that patients are getting the right drug, at the right price, and in the right setting has never been more important Accomplishing these goals, however, is challenging. Employers face complications in managing specialty drugs that do not exist for traditional medicines. A few key issues are highlighted in the following section.

Medical or Pharmacy Benefit

Because specialty drugs are often administered by physicians and require close monitoring, they are uniquely dispersed through two channels of administration and thus, two channels of reimbursement. Employees might receive specialty drugs through the pharmacy benefit, which generally covers outpatient prescriptions filled in drug stores or through mail order. Or, they might receive them through their plan's medical benefit, which generally covers drugs administered by a

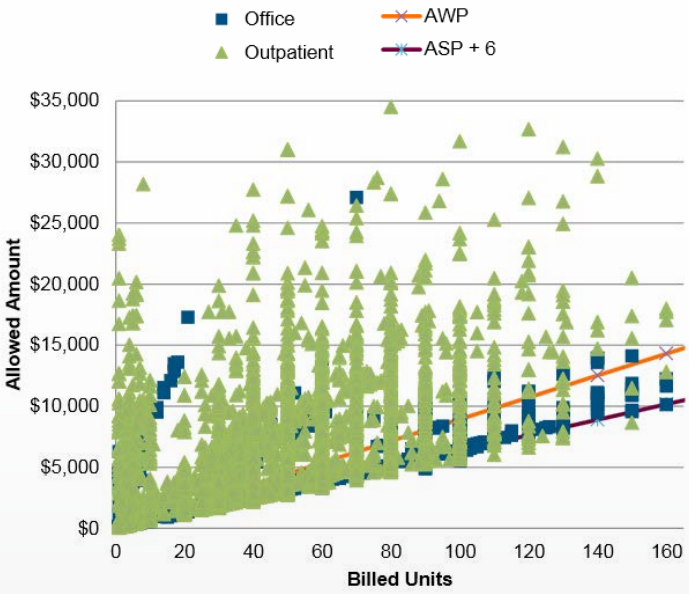
health care provider in an institutional setting, such as a doctor's office, a hospital setting, an infusion center or in their home by a nurse or other healthcare attendant.

If a medication is paid for through the medical benefit, a "J code" is recorded for the purposes of reimbursement —J codes are part of the Healthcare Common Procedure Coding System (HCPCS). Compared to National Drug Codes (NDCs), which are used when a medication is paid for through the pharmacy benefit, J codes are less informative and may include multiple products under one code. What's more, there is often a lag in assigning J codes, so new drugs may have an unclassified or unlisted designation.[17] Package size is not recorded through this code, and often, brand and generic drugs may share the same J code. These factors combined have the effect of substantially inflating overall expenditures for specialty medicines through the medical benefit, making it harder for an employer plan to manage specialty pharmacy benefits. An analogy from Managed Care Magazine explains, "If you need to track medication use and cost, J code data will give you a fuzzy picture; the NDC codes, a high-res one."[17]

Site-of-Care

Varying prices for health care services by site-of-care is not a new phenomenon, and prices of prescription drugs are not immune to these differentials. However, given the relatively high prices for specialty pharmacy products, ensuring that people receive them in the lowest possible cost setting becomes increasingly important. As noted earlier, many specialty drugs for the treatment of conditions like cancer, rheumatoid arthritis and multiple sclerosis are administered via infusion, which can be performed in a hospital, physician's office, infusion center or even the patient's home. The variance in price between different sites-of-care can be in the thousands of dollars,[18] underscoring the need to access pricing information so it will be possible to better understand if higher prices are associated with an increase in quality of care or improved patient outcomes.

Figure 2: Distribution of Remicade Allowed Cost by Claim Units



Data Source: *Milliman's HCG Commercial database. Graphic created by CVS Caremark.*

However, according to a 2014 survey of large employer groups, more than half (58%) had an understanding of site-of-service price differences related to specialty pharmaceuticals, but only 19% reported that they were able to steer patients toward high-value options.[19]

Adherence and Overall Spend?

Medication adherence is defined as taking medications according to the prescribed dosage, time, and frequency. Failure to follow prescription directions is known as failed adherence, also referred to as prescription drug waste. At least one study has closely tracked prescription drug waste in the United State, pegging the price of medication non-adherence at $269 billion dollars in 2013 alone.[20] The same study found that in addition to general waste due to non-compliance:[20]

- $55.8 billion was spent on higher-priced medications when lower-priced and clinically equivalent alternatives were available; and
- $93.1 billion could have been saved if patients had used alternative pharmacies[20]

Through our Institute on Health Care and Cost Solutions, the Business Group has worked with employee benefit managers, Pharmaceutical Benefit Managers (PBMs), pharmaceutical manufacturers, genetic/genomic diagnostic test manufacturers, and health plans to craft the following recommendations for employers to establish effective pharmacy benefit utilization management strategies:

- Implement a **comprehensive utilization management (UM) strategy** grounded in medical necessity/eligibility criteria.
- Implement a **prior authorization strategy** dictated by medical necessity/eligibility criteria.
- Implement a **step therapy program**, encouraging the use of lower-cost generics as first lines of treatment.
- Consider putting **quantity limits** in place to ensure proper dosage as well as patient tolerance and adherence.
- Consider formulary **exclusions or "exclude at launch"** strategies.
- Consider adding **additional specialty drug tiers** to your formulary to incentivize the use of lower-cost alternatives.
- Explore the savings potential behind **shifting certain specialty drugs from the medical to pharmacy benefit** to allow for greater visibility, better tracking data, more creative cost control strategies and increased oversight.
- Maximize savings by working with a **narrow network** of specialty pharmacies or by channeling patients through the most cost-effective specialty pharmacy network.
- Ensure that your PBM is keeping you apprised of potential changes and/or developments in the pipeline.

Additionally, the Business Group's National Committee on Pharmacy Benefits and Specialty Medicine focuses on ways employers can optimize these, and other strategies for specialty medication management. Members can visit our Specialty Pharmacy Management Series for an in-depth look at a variety of specialty topics of interest to employers.

Policy Barriers and Recommendations

There are many reasons for the high prices of specialty drugs, as well as underlying issues that make it difficult to develop strategies that will help lower these prices. This section explores these issues in detail and then identifies several steps that can be taken on the policy side to address them.

Foster Sustainable, Affordable Pricing of Specialty Medicines

In most other industries, prices are largely influenced by the traditional laws of supply and demand. Simply put, the value placed on goods and services by the customers has a significant role in determining market prices, and generally, the higher the price, the less product sold.

However, most experts believe that health care has an inelastic demand, meaning, those who are sick are not price sensitive. Moreover, since patients often pay only a fraction of the bill and they usually rely on their physician to determine what they need, demand is even less related to price in health care. Thus, given the nature of health care, its inelastic demand, third-party payment models and asymmetric economic information, it is hard for customers—patients, insurers, employers and governments—to determine value, let alone influence prices in a market-driven way. These phenomena disproportionately shift the influence for determining prices and defining value to providers and suppliers.

As they do in their own industries, employers recognize the significant investments drug makers commit to research and development. They also recognize that, as drug makers note, bringing a drug to market requires substantial capital and can take a decade or more, and that the vast majority of drugs fail. Furthermore, employers also recognize that, of the drugs that make it to clinical trials in humans, less than 12% secure full FDA approval.[21] Additionally, employers are even apt to accept that prices may be high for highly effective drug treatments, such as those for Hepatitis C, and that selected drugs may add value to the health care system by saving money.[10]

But along with patients and other payers, employers remain mystified by the incomprehensible prices of certain specialty drugs – both the initial prices and price increases for the same medications over time. This is particularly true for expensive drugs that do not provide a cure and may only offer incremental benefits. For these reasons, patients and payers are finding it increasingly difficult to understand and afford the prices.

While manufacturers contend that they conduct detailed valuation studies prior to making pricing decisions, other stakeholders suggest that prices are based on whatever the market will bear,[22] especially since demand for some therapeutic drugs is relatively inelastic.[12] Moreover, in addition to recouping the costs of research and development, many drug companies report significant profits—some as high as 50% or more.[23] Even if prices were cut significantly, there would arguably be sufficient incentive—in the form of reasonable profits— to continue to assume the risks of drug development.

Importantly, and as has been noted in this brief, the increase in drug prices is not limited to the launch of new drugs, but is aggravated by frequent price increases for drugs that have been on the market for many years. In addition, wide variation in prices for specialty drugs within and across sites of administration can tack on unnecessary and potentially exorbitant price inflators. The explosive growth in prices and spending for specialty pharmaceuticals has been alarming

for quite some time, but has grown increasingly so in recent years, accelerated by the increase in consolidation among medical providers, often referred to as vertical provider consolidation. This trend has contributed to growth in spending for specialty medications. While many industry experts suggest that vertical provider consolidation may lead to economies of scale and result in reduced spending overall, in practice it has often given hospitals and/or hospital outpatient departments (HOPDs) increased bargaining power in negotiations with payers, resulting in higher prices and/or changes in the mix of treatments provided to patients—and higher expenditures.[24] Further, this practice has led to a shift in specialty pharmacy reimbursement from the pharmacy benefit to the medical benefit, which has its own issues, as discussed earlier. **The Business Group believes that reasonable growth in prices and spending should track more closely with the rate of overall economic growth and wage growth to help ensure that employers, insurers, governments and patients have the means and the ability to pay for needed specialty pharmacy products.**

In the current environment, manufacturers often set prices unreasonably high initially and then frequently raise them faster than can be sustained by the overall economy and afforded by most people. These factors have raised political pressure to find alternative ways to price medications. With some recent high-profile cases of high prices for new medications and very high price increases for old medications, the public is ready for action. According to a recent study, "most Americans feel that drug costs are unreasonable (72%)."[25]

Given this growing public and political pressure, along with employers' growing concern over their ability to sustain trends in spending for specialty medications over the long term, **the Business Group believes it is necessary to review public policies that influence the pricing, prescribing and administration of specialty medications and recommends adopting and reinforcing new public policies that would create more sustainable, affordable pricing. Specifically, the Business Group recommends the following:**

1. **Remove Uncertainties Surrounding Risk-based and Value-Oriented Contracting and Implement Indication Specific Pricing and Reference Pricing in Public Programs**
2. **Limit the Reach of Medicare Part D Protected Classes**
3. **Eliminate Perverse Payment Incentives Under Medicare Part B**
4. **Encourage the Uptake of Biosimilars**
5. **Reform Permissive Patent and Exclusivity Protocols**

In the following section, each of these recommendations is explained in more detail.

1. Remove Uncertainties Surrounding Risk-based and Value-Oriented Contracting and Implement Indication Specific Pricing and Reference Pricing in Public Programs

How consumers use health care has changed significantly over the past several decades, but how they pay for services has not kept pace. Specifically, the fee-for-service model in which a set price is paid for a drug, irrespective of its health outcomes, is antiquated and inefficient. In the face of these outdated payment policies, industry stakeholders are already experimenting with innovative, value-oriented solutions, often thought of under an umbrella concept commonly referred

to as value-based payment (VBP) arrangements. VBP arrangements seek to more concretely tie payments to improved patient outcomes by implicitly tying reimbursement amounts to drug-associated patient outcomes and/or improvements in quality of life.

The following section describes different kinds of VBP arrangements and how they can be implemented.

Risk-Sharing Agreements

Within the umbrella concept of VBP agreements, payers and manufacturers are increasingly adopting risk-sharing agreements, in which manufacturers agree to reimburse or discount their products when the products do not work as intended. The Business Group recently compiled data on existing examples of innovative value-based and risk-sharing reimbursement models, which we believe hold great promise for more closely tying reimbursement policy to patient outcomes.

Excerpted examples of recent agreements include the following:

1. In 2015, **Amgen and Harvard Pilgrim Health** entered into an agreement for Amgen's cholesterol-lowering PCSK-9 inhibitor, Repatha.[26] According to media reports, in exchange for value-based reimbursement that is predicated upon both (i) patients meeting LDL-C reduction levels equivalent to the clinical trial outcomes, and (ii) patients taking no more than a set number of doses of the medication in a prescribed period, Harvard Pilgrim agreed to give Repatha unique formulary positioning.
2. In 2015, **Express Scripts** announced that it had entered into agreements with various manufacturers for various disease states in an effort to contract with manufacturers to set different prices for different indications based on relative value.[26]
3. In 2016, **Novartis** entered into pay-for-performance agreements with both Aetna and Cigna for its medication for heart failure, Entresto.[26] The agreements spell out financial terms tied to how well the drug improves patients' "relative health." The primary metric is reduction in the proportion of customers with heart failure hospitalizations. Aetna and Cigna will pay a discounted amount for Entresto if the medication reduces hospitalizations for their commercially insured patients with congestive heart failure. In exchange, Novartis will grow Entresto's market share through its position as a preferred drug, subject to prior authorization, on both Aetna and Cigna's formularies.

However, current policies inhibit the willingness of drug makers to enter into these types of arrangements on a full-scale basis, largely out of fear of their impact on current laws. Specifically, risk-based contracting, in which manufacturers share in the financial risk if medications do not work as intended, may trigger provisions of the Medicaid "best price" program. At this point, it is not clear how such arrangements relate to this policy rule.

In 1990, the Omnibus Budget Reconciliation Act established the best price provisions of the Medicaid drug rebate program. This law requires brand-name drug manufacturers to provide the Medicaid program with the lowest price they charge for any drug to any other payer. The intent of the law was to extend rebates manufacturers had previously negotiated with private plans in the Medicaid program. In practice, however, manufacturers abandoned some discounts, due to the market implications of a legislative mandate to extend them to the national Medicaid formulary.

The anticompetitive nature of the Medicaid best price program has been well documented by the U.S. Government Accountability Office (GAO), the Congressional Budget Office (CBO) and academic economists.[27] Apart from historical arguments against the best price policy, the program also imposes an impediment for drug makers to experiment with payers in new, innovative VBP arrangements because of the potential implications. Though manufacturers have increasingly shown a willingness to take on risk and potentially reimburse or rebate payers when a product fails to execute against pre-contracted outcomes, they have concerns that those rebates or reimbursements could trigger manufacturer obligations to Medicaid under the best price program.

Medicaid's Rebate Program

The best price, or the lowest price paid by any private-sector purchaser of a drug, including all discounts and rebates, is used to calculate the rebates that manufacturers are required by law to give to state governments for sales of brand-name drugs to Medicaid beneficiaries. Manufacturers must report the price to the Centers for Medicare & Medicaid Services in order to be paid for those drugs.

Rather than extending their best prices to the entire Medicaid market, firms have frequently chosen to raise their best prices.* Medicaid's rebate is equal to the greater of 15.1 percent of the average manufacturer price (AMP) or the difference between the best price and the AMP. In 1991, when the Medicaid rebate program was first implemented, the best price was, on average, 36 percent below the AMP for brand-name drugs. Since 1996, the best price has been a little more than 15 percent below the AMP, on average—roughly the level at which the best-price provision is triggered. For a brief period in 1991 and part of 1992, prices on the Federal Supply Schedule were included in the calculation of the best price; and during that period, many of the Federal Supply Schedule prices increased.** Legislation was then enacted to exclude Federal Supply Schedule prices from the calculation of the best price. More recently, to help Medicare Part D plans obtain lower prices; their negotiated prices were excluded from Medicaid's best-price provision under the Medicare Modernization Act.

The effect that the best-price provision has on the private-sector price of a drug (that is, the tendency to raise it) is greater the larger Medicaid's market share is.

* See Congressional Budget Office, The Rebate Medicaid Receives on Brand-Name Prescription Drugs (June 21, 2005).
** See General Accounting Office, Medicaid: Changes in Drug Prices Paid by VA and DOD Since Enactment of Rebate Provisions, GAO/HRD-91-139 (September 1991).

Source: "Prescription Drug Pricing in the Private Sector" (Congressional Budget Office), accessed September 19, 2016, https://www.cbo.gov/sites/default/files/110th-congress-2007-2008/reports/01-03-prescriptiondrug.pdf.

These types of risk-sharing agreements are a step toward creating a more sustainable pricing model for prescription drugs, particularly for expensive specialty medications. They distribute risks between payers and manufacturers more equitably and are likely to improve outcomes and the appropriate use of medications, resulting in improved quality. The arrangements also have the potential to create a win-win situation for both manufacturers and payers by offering incentives to employers, insurers and plan participants that all align toward appropriate, evidence-based use of expensive specialty medications.

We support the intent of risk-sharing agreements, as these types of interactions between insurers and manufacturers may have the effect of reducing prescription drug expenditures and overall medical costs for employers, although we recognize that more data are needed on the impacts. For these reasons, we believe it is critically important to remove barriers to VBP arrangements, particularly those constraining the creation of risk-based contracting.

Use of Value Frameworks

In addition to the possibilities for improvement offered within direct-contracting arrangements, another type of VBP centers around "value frameworks," which suggest ways to extend the dialogue about a medication's price and value as part of a shared decision-making model. Currently, there are four widely recognized value frameworks, which have been developed by the American Society of Clinical Oncology, the Institute for Clinical and Economic Review (ICER), the National Comprehensive Cancer Network, and Memorial Sloan Kettering Cancer Center. While each framework has different features, all the frameworks share one key goal: to encourage shared decision-making between providers and patients, particularly when choosing high-priced drug therapies.

Overview of Value Frameworks

AMERICAN SOCIETY OF CLINICAL ONCOLOGY
The American Society of Clinical Oncology (ASCO) established the Value in Cancer Care Task Force in 2013, which developed the framework. The ASCO framework "defines value as a combination of clinical benefit, side effects, and improvement in patient symptoms or quality of life in the context of cost." ASCO intends for the framework to be the basis for a software tool that doctors can use to assist shared decision-making with their patients.[28]
INSTITUTE FOR CLINICAL AND ECONOMIC REVIEW
The Institute for Clinical and Economic Review (ICER) has developed a conceptual framework for insurers to assess the value of medical services, including drugs, medical devices, and procedures.[29] The effort recently underwent a period of extensive commenting, which ICER intends to review and evaluate for a planned 2017 update.
NATIONAL COMPREHENSIVE CANCER NETWORK
The National Comprehensive Cancer Network (NCCN) has created five Evidence Blocks (Efficacy of Regimen/Agent, Safety of Regimen/Agent, Quality of Evidence, Consistency of Evidence, and Affordability of Regimen/Agent), with the goal of providing health care providers and patients the information necessary to make informed choices when selecting drugs, based upon measures related to treatment, supporting data, and cost.[30]
MEMORIAL SLOAN KETTERING CANCER CENTER
Peter B. Bach, MD, of Memorial Sloan Kettering Cancer Center, has developed an eight-domain strategy (efficacy, toxicity, novelty, R&D, rarity, population health burden, unmet need, prognosis, and annual spending) to attempt to put the cost of a drug in context with its overall value.[31]

Although value frameworks have potential, at this point in time, there is a lack of consensus around what constitutes value, especially when it comes to drug pricing. Dr. Bach notes, "It seems that there was a fair amount of coalescence around the possible domains of value, but there has been little effort and certainly little consensus on how much each of those domains should matter in the determination of value, or if they should even be included." In fact, according to a survey conducted by Avalere Health, out of 11 health plans surveyed, none were utilizing value frameworks.[33]

Nonetheless, employers are encouraged that risk-based and value-oriented contracting may help slow rapidly escalating health care spending. With that in mind, we expect to see more employers seeking opportunities to either directly engage in outcomes-based deals or encourage their plans to engage in these types of arrangements. We also encourage Medicare and other government programs to consider adopting them.

Other innovative approaches to ensure appropriate value payments for drugs may be to explore indication-specific pricing and reference pricing models in public programs, concepts the Business Group strongly supported in comments to the CMS on its proposed Medicare Part B payment demonstration. In our comments, we applauded the agency's willingness to evaluate the potential for value to be derived from indication-specific pricing and reference-pricing models and to use them for determining reimbursement.

Indication-Specific Pricing
This approach has the potential to increase payment alignment for a drug so that it corresponds to the value it delivers to a particular patient population: by adjusting reimbursement in accordance with the effectiveness of the medication for each indication.

With multi-indication drugs on the rise, many of which are high-priced specialty drugs, employers are interested in considering options that allow pricing to better reflect differential benefit by indication. In March 2016, ICER detailed[34] various models of indication-based pricing for pharmaceutical drugs, outlined the risks and benefits of these models for both payers and manufacturers, and made specific policy recommendations for how these types of agreements could be implemented. In particular, three major models of indication-specific pricing were described, which could be considered by policy makers:

1. Distinct product differentiation, authorized and marketed under different brand names with different prices;
2. No brand differentiation, but distinct, separate discounts applied for each indication; and
3. No brand differentiation; a single "weighted-average" price is developed using estimates of indication use across the population, with possible retrospective reconciliation through rebates based upon actual use.

Though employers recognize that there may be substantial implementation challenges to indication-specific pricing policies, we are encouraged by CMS's willingness to pilot this tool through the Medicare Part B program. In our comments, we recommended that CMS also maintain an open dialogue with employers and other payers, as well as with manufacturers and providers, to identify opportunities for additional legislative changes to federal reimbursement policies that obstruct indication-specific pricing agreements.

Reference Pricing
Employers have successfully implemented this policy, particularly when generic alternatives to more expensive brand medications are available and for specialty medicines, where there is documented price variation based on site of administration. As policy makers contemplate reference pricing policies for pharmaceuticals, one potential academic resource for reference is the Northwestern Journal of International Law and Business, which synthesized 16 studies

describing nine reference-pricing policies from six countries. The synthesis found that reference pricing "led to decreases in drug prices and increases in utilization of targeted medications, while also reducing payer and patient expenditures." The synthesis further suggested there was no increase in the use of medical services, such as physician office visits and hospitalization.

Recommendations for Policy Makers

- Consider exemptions for value-based contracts from Medicaid best price requirements and clarify how drug makers and payers can conceive of value-based contracts without triggering broader Medicaid best price program implications.
- Allow for variable pricing, where the price better reflects the evidence for benefit.
- Evaluate the usefulness and application of the existing developed value frameworks and their potential to impact drug pricing in public programs, as well as their overall utility to the health care system.
- Directly link reimbursement and improved patient outcomes.
- Consider how drug makers and payers can enter into other types of innovative VBP arrangements, such as indication-specific pricing.
- Implement reference pricing policies supported by clinical evidence consistently across public programs, where possible.

2. Limit Reach of Medicare Part D Protected Classes

Medicare Part D, also called the Medicare prescription drug benefit, is the federal program that subsidizes the costs of prescription drugs and prescription drug insurance premiums for Medicare beneficiaries. Anyone enrolled in Medicare—either Part A or Part B—is entitled to drug coverage through Part D, regardless of income. No physical exams are required, and applicants cannot be denied drug coverage for health reasons, because they have high utilization or for any other reason. In 2015, more than 39 million Medicare beneficiaries were enrolled in Part D drug plans. Since 2006, the share of Medicare beneficiaries enrolled in a Part D plan has increased, from 53% to 72% of all eligible Medicare beneficiaries.[35] Plans participating in Medicare Part D must offer either a defined standard benefit or an alternative benefit equal in value ("actuarially equivalent") or provide enhanced benefits.

While formulary design is a widely used private-sector tool for controlling private payer drug costs, Medicare has limited the freedom of Part D plans to control their formularies through specific rules, two of which substantially impact the price of drugs:

1. Federal regulations require that plan formularies include drug classes covering all disease states, with a minimum of two chemically distinct drugs in each drug class—a policy construct that allows drug makers to manipulate pricing based on artificial market share.
2. Plans are also required to cover all drugs in six protected classes: immunosuppressants, antidepressants, antipsychotics, anticonvulsants, antiretrovirals and antineoplastics.[36] What's more, CMS has gone beyond the statute, requiring at least one drug in each subclass as well.[37]

These rules limit the negotiating power of Part D plans and make drugs in those classes more expensive. Specific to the six protected classes, a Milliman study found that they accounted for between 16.8% and 33.2% of Part D drug costs.

The study suggested that reversing just this one rule could decrease prices in these classes by 9%–11% , for a projected Part D savings of $511 million per year.[37]

The Medicare Payment Advisory Committee (MedPAC), which provides independent, nonpartisan policy and technical advice to Congress on issues affecting the Medicare program and CMS, has twice recommended eliminating certain protected classes, but the proposals have been rejected both times.[38] This is unfortunate, because if adopted, MedPAC believes that the proposed changes to the Part D program could increase the payers' negotiating ability to lower prices of medicines in the protected classes.

This potential change would be significant not only for the Medicare program, but also for pricing in the commercial market, including for employer plans. Medicare's rules and pricing for prescription drugs influence system-wide resource allocation. Medicare costs extend well beyond the share of health expenditures it finances directly due to its large beneficiary base and sheer volume of transactions.[39] Medicare Part D payment and plan design policies for prescription drugs greatly affect the private sector. One author has likened this phenomenon to "bargaining in the shadow of a giant."[39]

According to a 2014 report, between 2006 and 2011, the prices for drugs in the six protected classes showed a trend similar to that for all Part D drugs, rising by a cumulative 28%, [39] driven primarily by two classes of drugs where generic competition was available: 1) antidepressant medications, which accounted for about half of the volume in the six classes; and 2) anticonvulsants, which accounted for about a quarter of the volume. Meanwhile, the same report notes that other classes made up almost entirely of brand-name drugs saw rapid growth in prices, ranging from increases of more than 30% for antiretrovirals to increases of nearly 80% for antineoplastics.[39]

When generic substitutions were considered, prices in protected classes fell by a cumulative 2% over the six-year period, signaling that plan sponsors had successfully moved enrollees toward generics when they were available. But, MedPAC further noted that the drugs' protected status may limit the number of rebates plan sponsors are able to obtain from manufacturers for drugs in these classes.[39]

Although there has been intermittent momentum to address the protected classes policy in order to save money in the Medicare program, there is no recognition by policy makers that current law limits private payers' ability to negotiate lower prices for certain drugs. And, despite its regulatory authority, CMS has been hesitant to implement changes that meet with opposition from drug manufacturers.

Recommendations for Policy Makers

- Following the independent MedPAC committee's recommendations, Congress and CMS should limit legislative and regulatory restrictions on formulary design within protected classes by modifying the Medicare Part D rules to remove drugs from those protected classes where sufficient generic competition exists, a change that would give private plans more freedom to control their formularies and negotiate for expanded manufacturer rebates.
- Specifically, CMS should resubmit its proposal to remove antidepressants, antipsychotics and immunosuppressants for transplant rejection from the list of protected classes because, in these classes, price reductions have been more closely linked to the availability of generics than to their status as "protected." CMS should stand firm against industry-funded campaigns that seek to undermine the agency's data-driven proposal to increase competitive pricing.
- At a minimum, policy makers should evaluate the potential anticompetitive influence of protected classes on the commercial market, and specifically, they should evaluate the limitations imposed on private payers' ability to negotiate competitive prices for drugs in the protected classes due to market spillover.
- Policy makers should work with stakeholders, including employers, to gain consensus for Medicare prescription drug policy changes that would remove additional hindrances to effective private payer pricing negotiation of these drugs. Then policymakers should work to implement those changes.

3. Eliminate Perverse Payment Incentives in Medicare Part B

As discussed earlier, most drugs in Medicare are reimbursed through Part D, its pharmacy benefit, but many specialty drugs are reimbursed through Part B, which is Medicare's medical benefit. This transactional difference takes place because specialty drugs often must be administered in a physician's office or hospital outpatient department. Because of this difference, Part B providers typically "buy and bill" for specialty drugs, meaning they buy the products in advance, store them according to the label specifications, and bill Medicare for reimbursement after administration to the patient. Provider reimbursement is calculated as ASP+6%, where ASP, or "average sales price," is calculated by CMS from manufacturer-reported prices for "sales to all purchasers," excluding sales that are exempt from Medicaid "best price" and sales at "nominal charge."[27]

This reimbursement model creates a three-part, cyclical incentive for prices to continuously rise. First, it encourages manufacturers to set prices higher and to incent providers to select those drugs—and receive a higher reimbursement. Second, it also creates an incentive for providers to continuously select higher-priced drugs, even when lower-cost alternatives might be available. Third, it incents the delivery of these medications in higher-priced settings, such as hospital outpatient departments.

We recognize and applaud that CMS has already shown a willingness to take on this issue in its proposed rule to reform the Medicare Part B drug payment model, and move from paying physicians administration fees based on a percentage of price to a set dollar amount.[40] In our comments to the agency, we were supportive in concept, but expressed concerns with the proposal as written, because it may have the unintended consequence of also accelerating market consolidation

and more vertical integration, particularly in oncology. Thus, we urge thoughtful consideration be given to balancing the elimination of financial incentives for prescribing a drug simply because its price is higher with the ability of non-hospital affiliated, independent clinical oncologists to absorb the financial impact, to avoid accelerated consolidation. In this vein, we feel strongly that manufacturer pricing of prescription drugs, a chief driver in expenditure growth in health care, should be the central focus of discussion, rather than physician reimbursement.

Recommendations for Policy Makers

- Eliminate financial incentives to providers who participate in Medicare to prescribe more expensive medicines, in more expensive settings; and,
- Encourage providers and manufacturers to assume financial risk with regard to high-priced drug utilization.

4. Encourage the Uptake of Biosimilars

Before turning to biosimilars, it's important to understand how biologics differ from traditional pharmaceuticals. A biologic is manufactured using a living system such as a microorganism, or plant or animal cells. Biologics, also referred to as "large molecules," are different from traditional pharmaceuticals, or "small molecules," in that the therapeutic target of a biologic is always a gene or a protein. Not all specialty medicines are biologics, but nearly all biologics are considered specialty medicines.

Historically, manufacturers of all biological products were required to seek FDA approval as if they were an entirely new entity, submitting a full complement of product-specific data, including animal and clinical study data. This approval design did little to encourage market competition among the highest-priced class of medications, even for similar products to treat the same diseases. But, the Affordable Care Act (ACA) sought to alter the landscape of biologics with the inclusion of the Biologics Price Competition and Innovation Act of 2009 (BPCIA)[iii] The BPCIA established an abbreviated approval

Biosimilar or Biosimilarity means the following:

- The biological product is highly similar to the reference product notwithstanding minor differences in clinically inactive components.
- There are no clinically meaningful differences between the biological product and the reference product in terms of safety, purity and potency .

Reference Product means the following:

- A single biological product, licensed under section 351(a) of the PHS Act, against which a biological product is evaluated in an application submitted under section 351(k) of the PHS Act.

Interchangeable or Interchangeability means the following:

- The biological product is biosimilar to the reference product.
- It can be expected to produce the same clinical result as the reference product in any given patient.
- For a product that is administered more than once to an individual, the risk in terms of safety or diminished efficacy of alternating or switching between use of the product and its reference product is not greater than the risk of using the reference product without such alternation or switch.

Definitions per "FDA's Overview of the Regulatory Guidance for the Development and Approval of Biosimilar Products in the US," accessed October 11, 2016. http://www.fda.gov/downloads/drugs/developmentapprovalprocess/howdrugsaredeveloped andapproved/approvalapplications/therapeuticbiologicapplications/biosimilars/ucm428732.pdf.

iii The Biologics Price Competition and Innovation Act of 2009 (BPCI Act) was part of The Patient Protection and Affordable Care Act (PPAC Act), signed into law by President Obama on March 23, 2010.

pathway for biological products that can be demonstrated to be "biosimilar" to and/or "interchangeable" with currently approved biological products, or reference products. The pathway created by the BPCIA is commonly referred to as the 351(k) pathway by industry insiders.[iv]

Biosimilars are often characterized as "generic biologics," because they must be certified by the FDA to have "no clinically meaningful differences in terms of safety and effectiveness from the reference product they were compared to." In addition, a biosimilar needs to have the same mechanism of action as the reference product it was compared to, which means it will work in the same way as the reference product.[41] In many ways, the BPCIA is to biologics as the Hatch-Waxman Act[v] is to traditional small-molecule drugs.

The abbreviated approval pathway permits a biosimilar biological product to rely on certain existing scientific knowledge about the safety and effectiveness of the reference product, saving the biosimilar manufacturer time and resources, thereby encouraging prices lower than those of the reference product. One estimate suggests that robust uptake of biosimilar products could reduce direct spending on biologics by nearly $45 billion by 2024,[42] by creating competition in a marketplace that has traditionally been anticompetitive. Thus, these products hold great promise to alter the trend on specialty pharmaceuticals, which is of particular interest to the Business Group. Our members are encouraged by the innovation taking place regarding biosimilars.

Biosimilars Market Overview – Four Approvals

Zarxio (filgrastim-sndz); brand: Neupogen (approved 3/6/15)
- Currently marketed in the U.S.

Inflectra (infliximab-dyyb); brand: Remicade (approved 4/5/16)
- Remicaid has been on the market since 1998 and had $6.6b in sales last year; in August, a judge invalidated an extended patent on the drug, which would have gone through 2027. The biosimilar plans to launch in October 2016, which will make it the first market monoclonal biosimilar.

Erelzi (etanercept-szzs); brand: Enbrel (approved 8/3/16)
- Enbrel has been on the market since 1998 and had $5b in sales last year; brand manufacturer argues it has patent life until 2029 and is in litigation with the biosimilar sponsor.

Amjevita (adalimumab-atto); brand: Humira (approved 8/23/16)
- Humria has been on the market since 2002 and had $12.5b in sales last year—the #1 selling drug in the world; brand manufacturer argues it has patent life until 2022 (at least) and is in litigation with the biosimilar sponsor.

Reference biologics in the United States are eligible for a 12-year market exclusivity period, which limits market competition and increases their pricing power.

iv Importantly, there is another approval pathway for a biosimilar, which is through the 505(b)(2) Pathway. The 505(b)(2) pathway is available for a relatively narrow category of biologics – specifically, those that had been approved under an NDA before the BPCIA was signed into law in March 23, 2010 – and it is only available for that narrow category of biologics until March 23, 2020. See § 7002(e) of the Affordable Care Act (ACA). Notably, any product approved under the 505(b)(2) pathway will be considered approved under the 351(k) pathway once the ten-year phase-in period is complete, according to § 7002(e)(4).("'Biosimilars' Under the 505(b)(2) Pathway," BIOLOGICS BLOG, accessed November 2, 2016, http://www.biologicsblog.com/blog/biosimilars-under-505-b-2-pathway-2/.)

v The Drug Price Competition and Patent Term Restoration Act (Public Law 98-417), informally known as the Hatch-Waxman Act, is a 1984 United States federal law which encourages the manufacture of generic drugs by the pharmaceutical industry and established the modern system of government generic drug regulation in the United States. Representative Henry Waxman of California and Senator Orrin Hatch of Utah sponsored the act.

But unlike the growth of the generic market after passage of the Hatch-Waxman Act, the BPCIA has not led to a flood of biosimilar approvals. Despite the landmark legislation, in the nearly seven years since its passage, there have only been four biosimilars approved by the FDA. By contrast, there are more than 20 approved biosimilars in the European market.

The Problem for Biosimilars and Competition in the Biologics Market

There are significant barriers to biosimilar development, which are much more difficult to overcome than barriers for the development of small-molecule generic drugs. Safety, pricing, manufacturing, market entry and physician and patient acceptance are all seen as tactical hurdles for stimulating competition in the biologics market.

Additionally, the FDA, which has regulatory authority for implementing the BPCIA, has been slow to offer guidance to industry on key provisions of the legislation, specifically for the naming and labeling of biosimilars, as well as defining what it means to be interchangeable. The FDA issued draft guidance for naming in August 2015 and draft guidance for labeling in March 2016, but as of this writing, neither guidance document has been finalized. On January 17, 2017, the FDA released guidance on the interchangeability of biosimilars. The guidance, which was originally expected in December of 2015, instructs that manufacturers seeking interchangeability designation should conduct one or more switching studies, between the reference product and the biosimilar, to demonstrate that patients can alternate between the two products safely and without diminished efficacy. Comments on the proposal are due by March 20, 2017, and a finalized rule is expected by the end of the year.

This uncertainty in the regulatory environment, especially regarding interchangeability guidance, has had the effect of slowing the entrance of biosimilars to the market. By establishing the separate and distinct interchangeable designation and then failing to clearly define its parameters for more than 6 years, the FDA has contributed to speculative patient fear around the safety of biosimilars. Indeed, *biosimilar* is a name meant to emphasize that even slight changes in production can result in a drug that is similar, but not exactly the same. Yet, despite the FDA's repeated and emphatic assertions that biosimilars are safe and effective alternatives to their reference biologics, the distinction between biosimilar and interchangeable is likely to have an impact on provider and patient acceptance.

Recommendations for Policy Makers

- Accelerate the definition of a dedicated regulatory pathway for biosimilar interchangeability.
- Work with stakeholders to disseminate provider and patient education to firmly establish the safety and efficacy of biosimilar drugs to their reference products, recognizing that key successes to the uptake of biosimilar medicines in other countries was predicated on the creation of trust and confidence among all the stakeholders involved, including prescribers, pharmacists and patients.[43]
- Maintain payer autonomy to implementing utilization management tools for specialty pharmaceuticals, including tools that pertain to biosimilar products.

5. Reform Permissive Patent and Exclusivity Protocols

After a generic or biosimilar is approved by the FDA, in many cases, it may still take years for the cheaper versions come to market. This is largely because of litigation brought by the manufacturer of the original drug, based on outstanding legal questions about whether the patents can be extended through various secondary approvals for the original drug. For example, the original patent for Humira, a biologic used to treat various types of arthritis, Crohn's Disease and other ailments, was set to expire in 2016, but its manufacturer has indicated that it has add-on patent protection from 70+ ancillary patents, which can extend the patent through 2022, and potentially beyond. These claims, however, seem to be unsubstantiated following an evaluation of the Patent Application Information Retrieval (PAIR) database housed at the Patent Trademark Office (PTO).[vi]

Why the discrepancy? Deciphering and understanding patent and exclusivity terms of pharmaceutical products is complicated because the two are intertwined and work in complementary, yet distinct, ways. And as these product protection terms have become increasingly important to market share and profitability, they are fiercely protected by the pharmaceutical industry, resulting in "patent estates," or "patent blockades," on top-grossing products.[44] These are multiple patents for one product, covering different indications, delivery methods, and/or combinations of the product. Thus, coming to an accurate determination of when a patent term expires often requires specialized legal expertise. A publication by the Center for Drug Evaluation and Research (CDER), part of the FDA, states that "patent" and "exclusivity" are two of the most commonly searched terms on the FDA website,[45] which underscores both the complexity and value of these product protections to drug manufacturers, as well as interest from outside stakeholders.

In a nutshell, market exclusivity is driven by 1) monopoly rights awarded following the FDA's approval of a new drug product and 2) the patents associated with the product.[46] Thus, drug makers' ability to sustain high prices in the United States hinges on the monopolistic character of the pharmaceutical market, driven by these patent and exclusivity protections, which insulate products from competition and artificially boost the industry's negotiating power.[46]

Patents vs. Exclusivity

As explained by the FDA, patents and exclusivity work in a similar fashion but are distinctly different from one another. Patents are granted by the patent and trademark office anywhere along the development timeline of a drug and can encompass a wide range of claims. Exclusivity is exclusive marketing rights granted by the FDA upon approval of a drug and can run concurrently with a patent or not. Exclusivity is a statutory provision and is granted to a New Drug Application (NDA) applicant if statutory requirements are met. Exclusivity was designed to promote a balance between new drug innovation and generic drug competition.

Patents and exclusivity periods were designed to reward drug companies for expensive innovations that benefit society. Unfortunately, the increasingly high prices that companies charge for drugs under this legal monopoly protection diminish the societal benefit of those medications.

A patent, at its most basic, is a property right to exclude others from making, using, selling, offering for sale or importing the claimed invention for a limited term. In general, pharmaceutical patents are considered utility patents, which are original inventions (other intellectual property protections include design patents, copyrights, trade secrets and trademarks).

vi Evaluation of image file wrapper for Humira, patent 08/599,226, at http://portal.uspto.gov/pair/PublicPair

Patents for pharmaceutical products work differently from those issued in most other industries, which will be explained further in this document. However, all patents are still issued by the PTO, and all initial patents provide 20 years of intellectual property protection. Importantly, when a patent is granted, the patent expiry is 20 years from the date of the original filing. However, specific to pharmaceutical products, there are two methods by which the original patent term may be extended. The first is through a "patent adjustment," which is intended to give time back to the applicant that may have been caused by delays at PTO. The second is through a "patent extension" (previously termed "patent term restoration"), which is intended to give time back to the applicant that may have been caused by delays in the FDA product review and approval process.

Patent Abuses and Anticompetitive Practices

The methods discussed above are the only ways that an original patent can be extended. Beyond these statutory extensions, the life of a drug's overall patent protection can be extended by applying for secondary patents through new formulations of the drug, new routes of administration, new indications, or uses of the drug in combination with another drug (see "patent estates" on pg. 22).

Members of the Business Group agree that an appropriate period of protection is essential to promoting investment in innovation and the discovery of new medicines, but we also believe a balance must be struck between both the right to enjoy the benefits as a creator of intellectual property and society's right to have affordable, adequate health and medical care. As mentioned previously, patents and exclusivity periods afforded to drug manufacturers by the PTO and the FDA are intended to reward innovators for their contributions. The expiration of patents theoretically yields generics and biosimilars, which benefit consumers. Unfortunately, what we sometimes see is repeated and dubious exploitation of the patent system, in which some drug makers game the process, thereby extending their monopoly market terms, which directly contributes to the unaffordable and unsustainable high-priced prescription drug market.

In the following sidebars, the implications of patent abuses and other anticompetitive practices are explained in more detail. While these practices do not in effect extend an original patent, though journalists often mischaracterize these as extensions, they do create patent estates, which increase the probability of litigation between branded and generic manufacturers and permit the branded manufacturer to continue to promote its product. Additionally, building these patent estates tends to run in congruence with applications for additional market exclusivity from the FDA.

PATENT ABUSES
"Evergreening" or "Product Hopping"
A practice in which a pharmaceutical company producing a brand-name drug makes minor or modest formulation changes that provide little to no therapeutic advantage to a drug's formulation for the purpose of extending the life of both patent protection and FDA exclusivity. Companies have been known to introduce a nearly identical version of a brand-name drug before patent expiration and allow the original brand-name drug's patent to expire, promoting the "new" drug as an improvement over the previous brand-name drug. The Federal Trade Commission (FTC) and other government officials have flagged this practice as anti-competitive, but it remains legal. In one case, the FTC said: "The very fact of product-hopping can itself be evidence of monopoly power. The manufacturer of a brand-name drug generally undertakes a product hop to preserve high profits that generic versions of the same drug would undercut but that no alternative drug, competing in the same market, has yet disciplined."[48] (See Table 2. In addition to using this method for piling up patents, FDA approval for a new use or a new formulation also triggers an additional three years of marketing exclusivity.)

Label Patents

Refers to any patent that covers a method or product recited in the FDA-approved label. Label patents typically target a new patient population or a new indication, or include a new dosage form, dosing regimen or route of administration. The patent term for such new patents is 20 years from date of filing, which can significantly extend the period of exclusivity for the repurposed drug.[49] Examples of label patents include:[49]

- Administering a different dose to the elderly;
- Titration of dosage over a certain number of days;
- Titration pack with escalation dosages;
- Administering a drug without food;
- Administering a dosage form that achieves plasma level of X, measured Y hours after dosing;
- Administering with an anticonvulsant in patients at risk of seizure;
- Informing the caregiver or patient to avoid taking the approved drug with another drug;
- Offering a drug in combination with unique packaging;
- Offering a drug in combination with a delivery device, such as an; and
- Providing a unit dosage of a drug with particular dissolution values or resulting pK values.

30-Month-Stay

Refers to burden placed on the generic drug manufacturer when bringing a potential generic entrant to market while operating under the assumption that a patent on the branded product is invalid; under those conditions, the generic manufacturer must file its application with a "paragraph IV certification," stating the reasons the patent is invalid. At this juncture, an automatic 30-month-stay is initiated, preventing the generic from coming to market to allow for the resolution of litigation between the brand and generic manufacturers. Improper patent listings by branded manufacturers trigger frivolous 30-month-stays; – this i practice has been flagged by the FTC and litigated extensively. Essentially, when this is done, branded manufacturers list new patents after an application for a generic is filed, which grants the brand-name company additional 30-month stays of FDA approval of the generic's application.

OTHER ANTICOMPETITIVE PRACTICES

Pay for Delay

There are two main types of pay for delay deals: 1) those involving cash payments from branded manufacturers to generic manufacturers to generic market entry; 2) pay- for- delay scenarios that occur when a generic company agrees to delay introduction of a generic version of a brand- name drug in return for the brand- drug-maker's agreement to refrain from marketing an authorized generic (a "no AG agreement") version of the branded product during the "first filer" 180-day exclusivity period, a practice that the FTC does not condone.

Patent Trolling

Because of the sizable amount of money involved in certain patents, some investment firms acquire many patents and then assert rights against branded manufacturers, sometimes in a frivolous manner. This practice is referred to as "patent trolling." The goal of many patent trolls is to extract cash settlements from manufacturers that want to avoid the expense of patent litigation.

Table 2: Overview of Patent and Exclusivity Provisions

TYPE	LENGTH	TYPE
Patent		
Patent	20 years	Original
Patent Term Extension	Up to 5 years	Extension to account for regulatory review delays
Patent Adjustment	Up to 5 years	Adjustment to account for patent review delays
Non-Patent Exclusivities for Pharmaceutical Products		
New Chemical Exclusivity (NCE)	3-5 years	Marketing & data exclusivity
Orphan Drug Exclusivity (ODE)	7 years	Marketing exclusivity
Biologic Product Exclusivity	4 & 12 years	4 years data exclusivity 12 years marketing exclusivity
First Interchangable Biosimilar (after reference Biologic patent expiration)	1 year to 42 months	Marketing exclusivity
"Other" Exclusivity (new use or new formulation)	3 years	Marketing exclusivity
Pediatric Exclusivity (can be used twice)	6 months	Marketing exclusivity
180-Day Exclusivity (for generics)	180 days	Marketing exclusivity

The costs of extended monopolies in the pharmaceutical market are more than just financial; they serve to threaten further innovation. Kenneth Arrow, a Nobel laureate in economics, has argued "that the incentive to invent is less under monopolistic than under competitive conditions but even in the latter case it will be less than is socially desirable."[50] Thus, policies that extend patent protection terms or exclusivity periods should be revisited by policy makers, as these anticompetitive practices not only reduce the pace of innovation, but are potentially significant causes of unaffordable and unsustainable drug pricing and ever-increasing spending. While this is not a new issue, it is one being used in the specialty market, already holding up three of only four currently approved biosimilar products.

Biosimilar Patent Dance

Two key provisions in the BPCIA that affect patents specific to biosimilars have ignited litigation—and delays in market entrance for competitor products. Both provisions have to do with the meaning of the word "shall."

The "patent dance" as it is known, was intentionally crafted within the BPCIA. The "patent dance" lays out the steps and schedule during which a biosimilar applicant and reference product sponsor exchange certain confidential information regarding the biosimilar.[51] This process also highlights the patents that may be the subject of future litigation between the two manufacturers. The reference product sponsor can assert any remaining identified patents once it receives notice from the biosimilar applicant 180-days prior the date it intends to market its biosimilar product.[51]

Two court rulings have defined how the patent dance proceeds. In the first ruling, for the provision, "Applicant shall provide application and manufacturing details after acceptance for review," the court ruled that "shall" means "may."[52] But in the ruling for the second provision, "Applicant shall give the Sponsor 180 days' notice before commercial marketing," the court ruled that "shall" means "must." [52] The combined result of these court rulings is an additional six months of exclusivity for biologic products, where biosimilar manufactures might argue that the intent of the underlying law was never to require an applicant to provide 180 days' notice prior to first commercial marketing, which cannot be given until the biosimilar applicant receives approval from the FDA.[53]

Recommendations for Policy Makers

- Reduce the market exclusivity for biologics from 12 years to 7 years.
- Eliminate or limit additive patent extensions and exclusivity periods that serve only to extend monopoly power, especially where there is limited or no additional company investment or patient value produced.
- Develop sound policy that would discourage patent abuses such as "evergreening" and "product hopping." These policies may include financial penalties, loss of exclusivity periods and/or reduced patent terms for other products.
- Eliminate pay-for-delay deals and/or implement penalty provisions for companies that engage in pay-for-delay deals.
- Refine the biosimilars patent dance to effectively incentivize the use of the section 351(l) patent dispute resolution provisions.

Reject Anticompetitive "Quick Fixes" that Would Further Harm the System

While employers and other payers support policy changes that encourage market-oriented solutions for managing high-priced specialty drugs, we are equally sensitive to resisting the urge for quick fixes. As consumers find themselves paying more of their drug costs, it's tempting to be lured into new policies, which would only further contribute to the anticompetitive climate. These policies may include specialty drug price caps, out-of-pocket payment caps, limitations on utilization management tools and mandated disclosure of propriety information.

These types of polices could induce various unintended consequences, including overpayments for mediocre drugs, drug shortages, making drugs less responsive to price, stifling innovation, undermining payer abilities to negotiate lower prices shifting higher prices to other payers, and raising premiums and health plan costs. More importantly, short-sighted approaches aimed only at immediate patient affordability miss the mark on establishing a long-term, sustainable pricing model.

Conclusion

In addition to implementing traditional techniques, including prior authorization, step therapy and utilization management tools, employers have also been on the forefront of developing innovative technologies to manage spending on specialty pharmaceuticals. These technologies have included building navigators and developing transparency tools to help employees evaluate providers and treatment options, determine value, access price and understand quality. But, employer efforts alone are not enough.

While plan design and utilization management tools can help drive down spending, policy reforms are needed to foster more sustainable pricing, promote appropriate utilization of high-priced drugs and reduce unnecessary spending. An effective public policy response to the substantial and ever-increasing growth in spending on specialty drugs will require open dialogue with all relevant stakeholders to construct market-oriented, consensus-driven strategies. Fundamentally, however, these strategies must focus on specialty drugs as a multifaceted challenge driven by 1) growth in specialty pharmaceutical development; 2) price and price inflation; 3) increased utilization for an expanded subset of conditions; and 4) the impact of vertical consolidation on specialty drug prices. Ultimately, employers are seeking a more rational approach to drug pricing, which will require an assessment of and modifications to a dysfunctional pricing process that undermines competition and inflates drug expenditures. As part of this reform, drug makers should consider more reasonable pricing because public policy reforms, employer plan designs and educational efforts, while necessary, are not sufficient for long-term sustainability of spending on specialty medicines.

Summary of Policy Recommendations

Remove Uncertainties Surrounding Risk-based and Value-Oriented Contracting and Implement Indication Specific Pricing and Reference Pricing in Public Programs

- Consider exemptions for value-based contracts from Medicaid best price requirements and clarify how drug makers and payers can conceive of value-based contracts without triggering broader Medicaid best price program implications.
- Allow for variable pricing, where the price better reflects the evidence for benefit.
- Evaluate the usefulness and application of the existing developed value frameworks and their potential to impact drug pricing in public programs, as well as their overall utility to the health care system.
- Directly link reimbursement and improved patient outcomes.
- Consider how drug makers and payers can enter into other types of innovative VBP arrangements, such as indication-specific pricing.
- Implement reference pricing policies supported by clinical evidence consistently across public programs, where possible.

Limit Reach of Medicare Part D Protected Classes

- Following the independent committee's recommendations, Congress and CMS should limit legislative and regulatory restrictions on formulary design within protected classes by modifying the Medicare Part D rules to remove those protected classes where sufficient generic competition exists, a change that would give private plans more freedom to control their formularies and negotiate for expanded manufacturer rebates.
- Specifically, CMS should resubmit its proposal to remove antidepressants, antipsychotics, and immunosuppressants for transplant rejection from the list of protected classes because, in these classes, price reductions have been more closely linked with the availability of generics than to their status as "protected" and stand firm against industry-funded campaigns that seek to undermine the agency's data-driven proposal to increase competitive pricing.
- At a minimum, policy makers should evaluate the potential anticompetitive influence of protected classes on the commercial market, and specifically, evaluate the limitations imposed on private payers' ability to negotiate competitive prices for drugs in the protected classes due to market spillover.
- Policy makers should work with stakeholders, including employers, to gain consensus around policy changes that would remove additional private payer hindrances to effective pricing negotiations of these drugs. Then policymakers should work to implement those changes.

Eliminate Perverse Payment Incentives Under Medicare Part B

- Eliminate financial incentives for prescribing more expensive medicines, in more expensive settings.
- Establish direct links between reimbursement and improved patient outcomes.
- Encourage providers and manufacturers to assume financial risk regarding high-priced drug utilization.

Encourage the Uptake of Biosimilars

- Accelerate the definition of a dedicated regulatory pathway for biosimilar interchangeability.
- Work with stakeholders to disseminate provider and patient education to firmly establish the safety and efficacy of biosimilar drugs to their reference products, recognizing that key successes to the uptake of biosimilar medicines in other countries was predicated on the creation of trust and confidence among all the stakeholders involved, such as prescribers, pharmacists and patients.
- Maintain payer autonomy to implementing utilization management tools for specialty pharmaceuticals, including tools that pertain to biosimilar products.

Reform Permissive Patent and Exclusivity Protocols

- Reduce the market exclusivity for biologics from 12 years to 7 years.
- Eliminate or limit additive patent extensions and exclusivity periods that serve only to extend monopoly power, especially where there is limited or no additional company investment or patient value produced.
- Develop sound policy that would discourage patent abuses such as "evergreening" and "product hopping." These policies may include financial penalties, loss of exclusivity periods and/or reduced patent terms for other products.
- Refine the biosimilars patent dance to effectively incentivize the use of the section 351(l) patent dispute resolution provisions.

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National Business Group on Health®

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