Specialty Pharmacy and Patient Care:

Are We at a Tipping Point?

AMCP FOUNDATION SYMPOSIUM

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INTRODUCTION

"Tipping Point"—"the critical point in a situation, process, or system beyond which a significant and often unstoppable effect or change takes place." (Merriam-Webster)

The potential for improved patient care and outcomes through the use of specialty pharmaceuticals holds great promise. However, this potential is facing intense scrutiny, primarily related to the cost and challenges of access to specialty pharmacy.

Edith A. Rosato, RPh, IOM, Chief Executive Officer, Academy of Managed Care Pharmacy (AMCP), stated that specialty pharmacy in general has produced innovation, better cure rates for patients with serious rare and chronic diseases, and often offers better tolerability over existing therapy. The higher costs commonly associated with these specialty products have caused much concern across the country, despite evidence that some of these therapies can be lifesaving (and many can potentially even be cost saving). Ms. Rosato, who is also chairman of AMCP Foundation, noted that in reaction to these higher costs, payers are employing benefit tools, including high co-insurance, which some believe go too far—preventing access to patients who can most benefit from the use of specialty medications.

Edith Rosato, RPh, IOM

The AMCP Foundation symposium, "Specialty Pharmacy and Patient Care: Are We at the Tipping Point?" examined the fundamental issues of access and affordability to specialty medicine. Moderated by John Mackowiak, PhD, Vice President of Pharmacy and Education, AMCP, and Editor-in-Chief of the *Journal of Managed Care & Specialty Pharmacy*, this symposium brought together payers, academics, and members of the pharmaceutical industry to discuss whether this point has been reached, and if so, where payers go from here.

The AMCP Foundation would like to thank the following sponsors for their unrestricted grants to support the symposium and this report monograph—the Amgen, Biogen Idec, National Pharmaceutical Council, and Pfizer Inc.

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STEPPING BACK TO LOOK AT SPECIALTY DRUGS

A report of a session presented by Robert W. Dubois, MD, PhD, Chief Science Officer, National Pharmaceutical Council

It does seem that we are approaching a tipping point in attitude toward specialty drugs, said Dr. Dubois, much as a result of the recent media attention to the launch and pricing of sofosbuvir (Sovaldi), as well as to providers' pushback to cancer medications that seem beyond patient affordability. Although he agreed that this spotlight is probably a positive overall, Dr. Dubois suggested that it may be a good idea to step back, before traversing this tipping point, and better understand the factors that drive it.

According to some analyses, Sovaldi is cost effective (based on a price discount), as Britain's National Institute for Health and Care Excellence (NICE) concluded. Although it is possible that the medication's cost effectiveness is the result of several health plans having negotiated discounts, Solvaldi is undisputedly a highly effective hepatitis C agent that can cure the disease with limited side effects, differentiating it from the previous standard of care.

This "attitudinal" tipping point is real, however. In analyzing how we arrived at this situation in time, said Dr. Dubois, four factors seem to have important bearing on whether we will step back from the edge or go beyond the point of no return.

Robert W. DuBois, MD, PhD

VALUE IS MULTIFACETED

Value is more than cost; it is composed of important clinical attributes like effectiveness and side effects, convenience in administration, and rapidity of effect—all compared with standard therapies. Many economists like to put a number on value, such as the incremental cost effectiveness, given in cost per quality-adjusted life-years. Dr. Dubois suggested that rather than seeking this simple relative figure, the value of a product should be dissociated, much like Zagat does for restaurant reviews. Preference comes into the equation, and a product's qualities or attributes are often in the eye of the beholder (e.g., its side-effect profile). It is possible that value may comprise both quantitative and qualitative components, in addition to a measure of cost per outcome. Dr. Dubois believes that contextual considerations should be an essential element in a payer's calculation of an intervention's value. For example, does the medication treat a life-threatening or less-serious condition? Having a different mechanism of action could yield additional benefits for some patients, he said. Having once-daily dosing may also prove advantageous for patient adherence. Finally, the calculation of value may be different for a payer than that of a health system or society as a whole. In the latter case, we must consider the ability to afford the intervention, based on limited resources. This does not, Dr. Dubois pointed out, mean that health system value equates to affordability—the other parameters still matter, but to differing degrees.

COMPARATIVE EFFECTIVENESS RESEARCH: A DOUBLE-EDGED SWORD?

Most payers agree that they want to see more comparative effectiveness research (CER), including head-to-head studies and trials comparing new treatments against practical treatments, not simply placebo. Furthermore, they are asking the industry for trials that don't use surrogate endpoints but specific clinical endpoints that are quantifiable over the long term and for trials with multiple patient subgroups. This has the potential to raise the cost of drug development, and possibly hinder innovation, according to Dr. Dubois. Payers may require CER for coverage,

limiting access to these medications. Therefore, CER may represent a double-edged sword, rather than a universal good.

An emphasis on CER can also have positive effects for the industry, including the use of new trial designs that actually cost less than conventional studies. Personalized medicine is helping to drive this, with its need for smaller patient populations. On the other hand, drug manufacturers' revenues can be positively affected through the higher justifiable price (though smaller population size) of personalized medicine, as well as the increased utilization of these agents based on the stronger evidence supporting their use. A study cited by Dr. Dubois indicated that CER may reduce innovation in certain disease states by up to 4.8%; although he pointed out that the confidence intervals were fairly broad in this evaluation.

"CER can encourage new trial designs that actually cost less than conventional studies"

COSTS SHOULD BE A LONG-TERM CONSIDERATION

The value of medications must be considered over the long term, said Dr. Dubois. This includes not only the long-term effects of the agent, but the long-term price as well. Consider the small molecule simvastatin (Zocor), the initial price of which was $1,500 per year. It is now available for far less today as a generic, meaning the value of the product has actually increased over time, particularly in clinical areas where the cost effectiveness was in dispute. It accumulates value over time, he remarked, so in a sense, today's small molecule generics pay for present and future biologic innovations. They are the funding source for the next wave of innovation. He believes that 10 years from now, a similar cost trajectory will be seen for biologics, owing to new processes to produce biologics in a less costly manner and the development of new, simple bioassays to determine the interchangeability of biosimilar competitors. Viewed only through the lens of today, according to Dr. Dubois, biologics would seem to be cost ineffective. This may be too short sighted, and he encouraged payers to consider the long-term picture.

BIOLOGY SHOULD NOT DETERMINE PATIENT COST

Payers are grappling with the higher costs of biologic drugs, and one way they try to manage them is by increasing patient cost sharing. However, Dr. Dubois believes that this results in inequities, based solely on a person's biology. Consider two patients with rheumatoid arthritis, both of whom take generic methotrexate. One person does well and continues to take methotrexate, but the other person does not receive sufficient benefit and the doctor prescribes a TNF inhibitor. This second person may end up paying a 20% co-insurance, whereas the other has only a generic copay.

If a companion diagnostic determines that a patient with metastatic colon cancer needs a specialty drug, should this patient have to pay more than the patient who does not need an expensive drug? Dr. Dubois said, "This is probably not fair, legal (from an antidiscriminatory standpoint), or economically efficient." Considering that the person who needed the TNF inhibitor was a "good soldier," it seems unreasonable that after first trying and failing a trial of the less-expensive medication, this patient should have to pay much more for treatment solely because of his or her own biology. On the other hand, if the difference in therapy was preference driven, such as for once-daily or oral therapy, differential cost sharing may seem more rational, according to Dr. Dubois.

Audience Participation

Time Compression and Plan Dollars. In discussing Sovaldi or any of the newer therapies to treat hepatitis C, one pharmacy director pointed out that "we're taking 20 or 30 years of underwriting and compressing it to obtain a measure of cost

effectiveness." He emphasized that the plan's time horizon is much shorter—the duration of that patient's membership in the plan.

Dr. Dubois responded that studies are ongoing at the National Pharmaceutical Council to try to determine who pays into the cost of these agents, who actually benefits from it, over what time period, and whether Sovaldi may be a unique case. The number of patients with hepatitis C infection today is huge, but he likened the situation to cataract removal in the Medicare population. Before cataract removal could be performed simply with lasers, cataracts were treated more infrequently. When laser procedures became available, Medicare was initially overcome with the number of procedures and claims. Today, the rate of cataract removal is down to the incidence rate of cataracts. Few are raising any eyebrows today about the procedure. He remarked that if Sovaldi cost $8,000 per year but had to be taken for 10 years, yielding the same total cost as today's regimen, few would complain. The issue is the compression of time.

"If Sovaldi cost $8,000 per year but had to be taken for 10 years...few would complain"

Incremental Benefits and Value. One audience member raised the issue of end-of-life care and the use of oncology agents that extend life by just a short period: How will we pay for this care? Dr. Dubois agreed that in order to pay for some new specialty drugs that are highly effective, "we'll have to kick some other stuff to the side that are not of great benefit." He added that we need to have an honest dialogue about end-of-life care with the patient and family, saying "death panels be damned"—we have to talk about it.

What the Market Will Bear. A health plan medical director commented that because Sovaldi is highly effective and should result in fewer cases of cirrhosis and transplants, a relatively high price may be justified. However, he affirmed that it would be helpful to have transparency in pricing, or justification of a price tag of $1,000 per pill. This also applies to oncology products. "It just seems to be based on whatever the market will bear," he said.

Dr. Dubois responded that transparency in pricing is desired not just for the pharmaceutical industry but for all manufacturers (e.g., Apple). He also pointed out that, as was the case of Sovaldi, if the National Institutes of Health, a public institution, was involved with the basic science, not the drug company, then it would make sense that this should also be considered in the price.

THE TIP OF THE ICEBERG

A report of presentations by Matt Salo, PharmD, Executive Director, National Association of Medicaid Directors and John Rother, JD, President and CEO, National Coalition on Health Care

THE HIGH COST OF CURING HEPATITIS C: A VIEW FROM MEDICAID

Medicaid is the largest insurance program in the country, and it is growing. In 2013, before the expansion instigated by the Affordable Care Act, Medicaid covered 72 million Americans. "It is the duct tape of the health care system," said Matt Salo. "Things that other health care payers won't deal with, we do. It is Medicaid that pays for the vast majority of the long-term care. Medicaid pays for the majority of mental health treatment and HIV medications in this country. That's an important preface to this discussion, because when it comes to cost and expense, this is kind of our bread and butter."

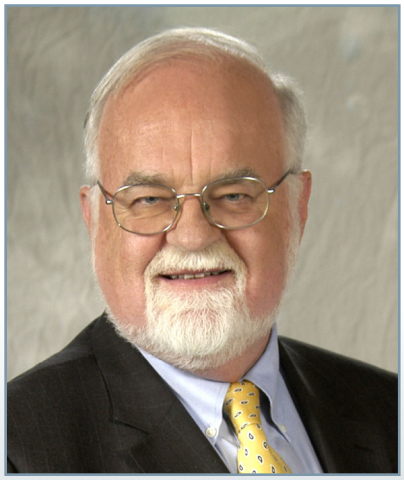
If unable to bring costs down to manageable levels, Mr. Salo believes states will employ more aggressive utilization management tools.

He stated that Medicaid directors have to be wise stewards of taxpayers' dollars, and they make decisions every day about prioritizing many underfunded needs. Medicaid budgets are finite, set by state legislatures. As a result, the high cost and widespread need for Sovaldi and medications like it in the Medicaid population pose a unique challenge to limited budgets.

Dr. Salo emphasized that high-cost therapies, whether for multiple sclerosis, cystic fibrosis, or cancer, are concerning for Medicaid, but the hepatitis C scenario presents a new and more difficult scenario—the combination of cost and exposure. For example, although Kalydeco treatment for cystic fibrosis may cost $200,000 per year, the number of people with the disease is very small. On the other hand, the number of Medicaid patients with hepatitis C may be 750,000. Dr. Salo remarked that some states are saying, "if this is the price we're going to pay, coverage of Sovaldi could double our pharmacy spend." He added, "Because of the nexus of cost and exposure, we will be paying much more attention to every new drug coming down the line. Awareness has been raised as to the proper price point, the definition of value, and how we deal with this. This is an issue that is being confronted at the state level right now."

State Medicaid programs have some tools at hand to control their costs, explained Dr. Salo. First, they try to negotiate the price down on the front end and offer a high rebate on the back end. If unable to bring costs down to manageable levels, he believes states will employ more aggressive utilization management tools. That means finding the "most thoughtful way possible" to prescribe fewer units of drug. He acknowledged that these tools will be neither sustainable nor effective. The resulting effort to prioritize the most appropriate candidates for treatment may exclude some groups, like current intravenous

Matt Salo, PharmD



John Rother, JD

drug users. If individuals start suing for access to these agents, said Dr. Salo, "I don't know that we will win those battles in the courts."

He emphasized that if state Medicaid budgets balloon over the short term, it can have very serious repercussions elsewhere. The legislatures will not increase their budgets to cover the overages, he said, and federal action, like additional risk corridors or even price controls, may be necessary in the short term to resolve this problem.

As the pharmaceutical pipeline is full of innovative yet likely expensive treatments, broader national debates will be needed to help focus the nation's priorities. "We may deal with the Sovaldi issue, but we won't be out of the woods," concluded Dr. Salo. "This is just the start. We need a fundamental debate in this country about the cost versus value of these interventions."

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EMPLOYER AND PRIVATE PAYERS: FINDING COMMON GROUND IN ADDRESSING SPECIALTY DRUG SPENDING

In the private sector, we're a little more immediately constrained than Medicaid, said John Rother, and at the National Coalition on Health Care (NCHC), "we've been focusing on the issue of affordability since 2011." His organization believes that about 30% of the health care dollar, covering many areas of administration and care, does not contribute to improving health. "Our mission is to get us to a higher value, better outcome system," Mr. Rother stated, "through changing incentives for physicians and providers, increasing the amount of transparency in the system, and incorporating greater competition."

Sovaldi was the trigger to a reevaluation of drug spending, he noted. Only one year ago, overall drug spending was considered stable, despite the considerable pipeline of investigational specialty drugs. Yet, NCH members began to air their concerns that a big problem now exists, and Sovaldi was the focus of the problem in terms of awareness and cost.

Partly, Sovaldi triggered outrage among payers, physicians, and employer groups because the molecule was developed at the National Institutes of Health, explained Mr. Rother. The lead researcher left to start his own company, which was subsequently acquired by Gilead. The projected cost of Sovaldi was purported to be $34,000 per treatment before Gilead's purchase. The final retail price of $84,000 did not seem transparent or reasonable to payers—it seemed unrelated to common pricing philosophy and the fact that so many people may benefit from it. Mr. Rother noted that the FDA's new approval of the all-oral combination drug will only further increase demand, at a potentially higher price.

Although Gilead claimed that the pricing for Sovaldi was based on value, many believe that it is founded on the previous standard of care, with a substantial price increase. Mr. Rother pointed out that this is inherently inflationary and that "over time, we cannot afford subsequent new products to be priced in this manner. This is why we saw *60 Minutes* air its recent story on drug pricing." Perhaps there was consideration of the fact that the agent actually cures hepatitis C and does so rapidly and with fewer side effects than ribavirin and interferon therapy; however, lack of transparency clouds the issue.

Today, Capitol Hill is questioning what can be done to head off the problem. Mr. Rother believes

that Sovaldi is just the beginning—"it's the canary in the coal mine." Specialty drug spending is growing, driving projected increases in overall drug spending. Seventy percent of new drugs approved by the FDA in 2013 were specialty pharmaceuticals. According to manufacturers, this increased spending will be offset by corresponding savings in other parts of the health care system.

Specialty drugs present tremendous cost challenges for employers. It is expected that specialty drugs will account for 40% of total pharmacy costs by 2020, up from 17% in 2014. Specialty drug spending may account for $845 in per-member per-month cost by 2018. The employers' responses have been to move to high-deductible plans and increased copays, shifting the costs to employees. As copays go up, Mr. Rother said, most Americans will not have the ability to pay for these agents, with implications for patient access and adherence. With workers' take-home pay being relatively flat, and their share of rising health care costs increasing, they will find it more difficult to afford quality care. For employees and their families, value is a greater concept than simply health care. Rising health care costs mean tradeoffs in other facets of life, which they may not be willing to make. Mr. Rother pointed out that ultimately, the competitiveness of U.S. businesses will suffer.

Partly, Sovaldi triggered outrage among payers, physicians, and employer groups because the molecule was originally developed at the National Institutes of Health.

To address the high costs of specialty pharmaceuticals overall, plans have taken numerous actions:

- Integrating pharmacy with medical benefits
- Increasing cost sharing
- Seeking ways to increase adherence with therapies
- Contracting with specialty pharmacies
- Engaging in pharmacy and utilization oversight

Mr. Rother favors approaches that utilize medication therapy management to more fully engage the patient in clinical care, while ensuring patient access and adherence. Cost shifting to workers has implications for patient affordability and adherence. He also advocates for employers to join coalitions "to hold specialty drug manufacturers accountable, before the public and Congress, for unreasonably high price setting that can limit patient access and threaten the sustainability of the health care system." Mr. Rother also recommended that greater price transparency would be better for all stakeholders, with a longer period of notification before launch of what that price will be, to enable payers more time to prepare.

He also suggested that an organization like the Government Accountability Office (GAO) could analyze the financial implications of high-cost drugs across the health care system and for the nation, serving as the "umpire of value." Other suggestions included re-introducing ideas that were originally discussed, but not included, in the Affordable Care Act. These included a longer period of manufacturer exclusivity, removing the physician incentive to prescribe higher-cost Medicare part D drugs, and expediting approval for competitor agents when an innovator drug's approval was expedited. Mr. Rother also believes that the role of the Patient-Centered Outcomes Research Institute (PCORI) should be expanded to justify the benefit of every new technology introduced. He agreed that nonpartisan, credible groups should be charged with conducting cost-effectiveness analyses.

Finally, Mr. Rother believes that we should look for new ways to finance health care innovation, other than through health insurance premiums. He suggested basing payments on the outcomes associated with agents, not the cost of development, in a risk- or outcomes-based format.

Audience Participation

Is a Single-Payer System Next? As the discussion turned toward new mechanisms of financing care, the long-discussed question was raised: Is the nation more willing to discuss a single-payer system today than in the past? Although the case of hepatitis C may be just the tip of the iceberg, Mr. Salo noted that this is still not a politically viable solution.

Mr. Rother pointed out that Medicare is a single-payer system, outside of the drug benefit. "No part D drug program has any leverage over prices," he said. Neither Dr. Salo nor Mr. Rother would not rule out the possibility of government-based price controls, however, should the current unsustainable trends continue.

Postlaunch Price Increases. Not only are initial retail prices for specialty drugs concerning, according to one pharmacy director, but price increases for agents after launch have also raised eyebrows. Mr. Rother responded that these actions are inherently inflationary and are often based on actions of other pharmaceutical manufacturers. "Price increases cannot be based on existing therapy," he said, "but this will require changing thinking and behavior across the industry."

Recommendations for Trade Groups. In the face of these issues, one pharmacy director asked, how can our trade and professional groups get more engagement and visibility around this issue? According to Mr. Rother, "It's time for employers, patient advocates, and state governments to speak up so our federally elected representatives take it more seriously than they have in the past."

As copays go up, Mr. Rother said, most Americans will not have the ability to pay for these agents, with implications for patient access and adherence.

Dr. Salo repeated that change on Capitol Hill is a slow process. However, we may be nearer today than ever before. "The more the conversations are heard, the closer we come to that tipping point," he said. "We're not saying that profits are a bad thing, but my perspective is from a closed system [Medicaid]." We need to incentivize innovations, but we need to balance that with limited funds. "We can do it thoughtfully," he cautioned, "or we can do it in a very dangerous way."

POTENTIAL ROLE FOR VALUE-BASED INSURANCE DESIGN IN SPECIALTY PHARMACEUTICALS

A report of a presentation by A. Mark Fendrick, MD, Director, Center for Value-Based Insurance Design, and Professor, Division of General Medicine & Health Management and Policy, University of Michigan

The reason we go into pharmacy or medicine, began Dr. Fendrick, is not to save money but to help patients. However, "regardless of how we can improve outcomes in medicine, the conversation is always about how much we're spending on these interventions." He pointed out that there is systemic underuse of the things that work best in health care. Instead of talking about spending fewer health care dollars, he believes we should turn the conversation around: The discussion should not be on how much we spend but on how well we spend. How do we spend more on the high-value interventions?

THE ROLE OF CONSUMER COST-SHARING IN MEDICAL DECISIONS

Archaic one-size-fits-all cost sharing fails to acknowledge the differences in clinical value among medical interventions, according to Dr. Fendrick. Instead, copayments and co-insurance should encourage consumers to use health services appropriately and emphasize the use of high-value services. "How can we justify in a preferred branded tier that lifesaving drugs cost the consumer as much as branded non–lifesaving drugs?" he asked. Unfortunately, consumer cost sharing is rising in this one-size-fits-all scheme.

A. Mark Fendrick, MD

A decade ago, the emphasis was to give consumers "skin in the game," to encourage them to use health services more appropriately. However, it makes little sense to raise consumer cost sharing for products that clinicians and policymakers want patients to take. Common sense dictates, and studies show, if costs are higher for a product, patients will use less of it. Cost sharing leads to a reduction in the use of essential care, which worsens health disparities, and in some cases leads to greater costs, noted Dr. Fendrick. This applies to mammography, flu shots, as well as medications for chronic conditions.

The prevalence among the sickest elderly (those with ≥ 4 chronic conditions) of cost-related nonadherence actually rose from 14% in 2009 to 17% in 2011, which countered previous downward trends. The prevalence among the sickest elderly of forgoing basic needs in order to purchase medications decreased from 9% in 2007 to 7% in 2009, but it jumped to 10% in 2011.

Dr. Fendrick pointed out that this applies to office visits as well as services. When copays rise, patients see their primary care physicians and specialists less often. For patients in Medicare Advantage plans, ambulatory physician visit copays nearly doubled for primary care and climbed more than 80% for specialist care in just a few years, while remaining unchanged in the Medicare fee-for-service program. In the year following these copayment increases, there were 19.8 fewer annual outpatient visits per 100 Medicare Advantage enrollees, with 2.2 additional hospital admissions per 100 enrollees. Authors of this study found even greater variations in vulnerable patient populations.

THE "WALL STREET JOURNAL OF MEDICINE," PAYERS, AND VALUE-BASED INSURANCE DESIGN

Dr. Fendrick believes that corporate employers are the greatest driver of change in health care.

IBM stopped requiring its employees to make a $20 primary care copay to encourage office visits. Others may follow the lead from business, he suggested. When a critical mass of employers endorse new policies (i.e., another tipping point), consumers and politicians generally follow.

This action by IBM is part of a greater trend toward value-based insurance designs (VBIDs), which set consumer cost-sharing levels according to the clinical benefit, not the acquisition price, of the service. In the case of IBM, this employer believed that primary care office visits led to positive, cost-effective outcomes, and were to be encouraged. Applied generally to medical and pharmacy benefits, VBID reduces or eliminates financial barriers to high-value clinical services and providers.

Value-based insurance design has been implemented by public and private payers, and

"The discussion should not be on how much we spend but on how well we spend. How do we spend more on the high-value interventions?"

Dr. Fendrick reported evidence showing that VBID improves adherence by an average of 5% and lowers consumer out-of-pocket costs, without increasing total spending by a significant amount (pharmaceutical costs generally do rise). However, improvements in medication adherence are extremely difficult to attain. "Even when we make the drugs free, we may raise adherence 25%," he said. "But the absolute numbers are still low." However, another positive result of VBID is that plans employing this policy tended to focus efforts on high-risk members, avoided disease management programming, and exerted greater influence on adherence than plans not employing VBID.

Dr. Fendrick introduced a new term for refining VBID and health policy discussions: *clinical nuance*. He explained that the extent of clinical benefits from a specific service depends on who receives it, who provides it, and where it's provided. Clinical nuance implies that cost sharing should be based on the clinical benefit produced from the intervention. For example, a person who is a first-degree relative of someone with colon cancer "should get paid to be screened." For persons over 50 years of age, colonoscopy should be free of cost sharing. On the other hand, if someone is not at risk and is only 40 years old, "that person should be fined for getting a colonoscopy, and pay 100% of the cost of the test."

APPLICATION TO SPECIALTY PHARMACY

In specialty pharmacy, the medications are more expensive, as are the patient co-insurances or copayments. From a VBID perspective, the patient cost share for a cancer drug that cures the disease 90% of the time should be less than for a medication that never cures a cancer but prolongs life for a month or two. Dr. Fendrick also believes that patients who complete the plan's step-therapy protocol before using a specialty pharmaceutical should pay significantly less than someone who uses the same medication as first-line treatment. "Only 30% to 35% of patients with rheumatoid arthritis receive sufficient improvement on methotrexate alone," he said. "Why do they have to pay a much higher cost for a TNF inhibitor?" It makes little sense to have a one-size-fits-all cost-sharing policy. Dr. Fendrick noted that these are "low-hanging, high-value fruit," which exist through much of specialty pharmacy today.

He recommended that payers impose no more than modest cost sharing on high-value specialty pharmacy, based on clinical nuance. This may mean different levels of cost sharing depending on

where an infusible product is administered, as well as in accordance with patient- and disease-specific characteristics.

USING CLINICAL NUANCE TO ALIGN PAYER AND CONSUMER INCENTIVES

Clinically nuanced VBID has been successfully implemented by hundreds of public and private payers, Dr. Fendrick reported. Value-based insurance design "version 3.0" not only focuses on incentives for patient cost sharing for drugs, but also restructures many of the supply-side provider incentives through payment reform (global or bundled payments, pay-for-performance, and accountable care), thus encouraging the use of high-performing providers through tiered networks, and using health information technology to identify high-risk patients.

It incorporates value-based cost-sharing policy based on:

- Prevention/screening
- Diagnostic tests/monitoring
- Treatments
- Clinician visits
- Physician networks
- Hospitals

Dr. Fendrick noted that provider-focused initiatives (i.e., supply side) alone have historically paid little attention to consumer decision making (i.e., demand-side behavior). Adding clinical nuance to payment reform and consumer engagement initiatives can help align payer and consumer incentives, he stated. "Aligning the supply- and demand-side incentives can improve quality and achieve savings more efficiently than either alone."

A VBID FUTURE

According to Dr. Fendrick, VBID has bipartisan political support as well as support from multiple public and private stakeholders. He helped write Section 2713 of the ACA, which selected preventive services to be provided without cost sharing. Clinical nuance is now being considered in VBID efforts and proposals are circulating in Congress that include greater use of VBID in the Medicare program.

There are still barriers to VBID in some areas, including rules applying to health savings account–qualified high-deductible health plans. Specifically, consumers must spend their entire deductible in these plans before being eligible for free value-based preventive services. The Internal Revenue Service is addressing this, according to Dr. Fendrick.

Audience Participation

Not Free to Consumers. What are the "right" levels of cost sharing for consumers that will encourage access to high-value interventions and discourage the use of low-value services, while not putting an undue burden on the patient? Dr. Fendrick said that he is a big proponent of consumer cost sharing overall, but there is no perfect answer. He would prefer the use of high levels of cost sharing on the clinical services for which evidence is lacking or found to have relatively little value (or even harm). Most important, though, is encouraging the use of high-value services, which will not save money but will make consumers happy. To make the plan CFO happy, you'll have to cover these high-value

services but also employ the other provider-based aspects of VBID. The key is to make members think twice about obtaining services that have low value or are supported by little evidence.

Manufacturer Coupons. A pharmacy director from the pharmacy benefit management industry said that manufacturers are the other party at the table. If payers create a benefit design that discourages the use of low-value, high-cost drugs with higher cost sharing, manufacturers often provide copayment coupons that help the patient avoid or eliminate that cost, essentially undercutting the benefit design. Dr. Fendrick responded that a new study in *Health Affairs* found that $250 is the threshold amount for specialty drugs at which cost-related nonadherence becomes a significant issue.

He also pointed out that copay coupons do not consider clinical nuance. They are provided regardless of whether the patient has gone through other step therapy first, whether he or she is receiving the specialty agent in the appropriate setting, or whether they are appropriate candidates to receive the drug. Dr. Fendrick indicated that he is not a supporter of one-size-fits-all coupons; however, he would support clinically nuanced couponing, if managed care pharmacy does not step up to meet the need for access. He reiterated that there is still considerable underutilization of low-cost, high-value drugs, and the industry can do more to encourage use of these agents.

ENHANCING VALUE OF SPECIALTY PHARMACEUTICALS: LESSONS FROM A CONSUMER ADVOCATE

A report of a presentation by N. Lee Rucker, MSPH, Principal and Founder, Enhance Value

Ms. Rucker's organization, Enhance Value, seeks to advance the discussion on value through three concepts: (1) Trust but verify, (2) keep it simple, and (3) get each stakeholder to share accountability for value.

TRUST BUT VERIFY

Trust is an essential element of the relationship between patients and physicians, and consumers and medicine, said Ms. Rucker. Yet today, patients can easily seek care outside of an existing patient–physician relationship (through urgent-care clinics or minute clinics). Trust is not necessarily a strong characteristic of these nontraditional office visits. Specialty drugs are most likely to be prescribed within a trusting, existing patient–physician relationship.

When patients were asked about the most important characteristics of their health care providers, a 2014 survey reported that being treated with honesty and respect by their providers (which engenders trust) is at the top of the list. In comparison, providers' use of treatment guidelines was rarely mentioned.

N. Lee Rucker, MSPH

Trust is only one aspect in prescribing specialty drugs. "If trust is truly established, what is there to verify?" she asked. "A lot [for drug treatment] and the list is growing." Not only does this entail the appropriate (nonbiased) prescription of a small molecule or specialty drug, but verification also includes gauging patient preferences. That is, do they prefer prescription drug therapy or nonprescription interventions? If they prefer prescription treatments, do they prefer traditional or specialty, and what is most appropriate from a clinical standpoint? Can the patient afford the out-of-pocket costs? Also, what is the responsibility of the patient to get the most out of the therapy prescribed? This is also subject to verification (i.e., adherence).

The evolving base of evidence is very difficult for patients as well as some practitioners to comprehend. Ms. Rucker asked, "Are prescribers ready to have evidence-based conversations with patients and caregivers?" Is the level of trust in the existing patient–physician relationship sufficient for the physician to tell the patient "no" if appropriate? This is evolving, according to Ms. Rucker, from the perspective of balancing patient expectations and the level of evidence to support use of a specialty product, in addition to aligning "evidence-based medicine with patient-centered care."

The Center for Advancing Health advocates that patients must take it upon themselves to gather additional expert opinions and ask about the evidence for efficacy and safety of recommended treatment options. Patients must be engaged in negotiating a treatment plan with the provider, based on their own preferences. Ms. Rucker emphasized that we need to encourage patients to become more engaged, to help them choose high-performing providers based on evidence-based clinical pathways. She also pointed out that "there is a disconnect for patients and providers. It may be growing with new drugs, and conversations need to be started as early in the process as possible."

KEEP IT SIMPLE

Ms. Rucker advised providers to keep these conversations simple, using language that engages the patient. Some examples are as follows:

- "The evidence shows that treatment X works best in this type of patient..."
- "Tell me about your preferences. What are your main goals of treatment? What do you want to avoid?"
- "In treating patients like you, my experience has been..."
- "Here's what I would recommend..."
- "How does that sound to you?"

Although the patients in the aforementioned survey gave evidence-based guidelines low priority, the story is quite different for engaged patients—that is, patients who have done their homework and understand a good deal about their treatment and provider choices. Ms. Rucker indicated that quality and process-related options matter to them: the use of clinical decision support (e.g., clinical oncology pathways); better integration and use of patient registries and their associated, highly relevant, patient-reported outcomes; and the use of quality measures that reflect care processes. "What matters to engaged patients," she said, "could benefit all patients." Ms. Rucker also believes that emphasis should be placed on quality measures that are more general (less disease specific) and would promote optimization of medication use."

SHARED ACCOUNTABILITY FOR VALUE

According to several studies, 75% of all office visits conclude with a prescription, and perhaps 4 of every 100 office visits for a commercially insured population conclude with a prescription for a specialty medication. An Express Scripts study found that specialty drugs accounted for approximately 70% of all drugs approved by the FDA in 2013, compared with 33% in 2008.

Part B expenditures for specialty drug use are increasing as well (approximately $13.2 billion in 2012). The top 5 specialty drugs account for 30% of Part B spending (most of which paid for infusion drugs for cancer and autoimmune disease, and injectables for eye care).

Part D spending on specialty drugs is even greater, said Ms. Rucker. Even though less than 1% of medications cost more than the $600-per-month cost threshold to be designated for the Part D specialty tier, 11% of all Part D expenditures were for specialty tier drugs. Patients in the low-income subsidy group account for nearly 80% of total part D costs, she pointed out. The Centers for Medicare and Medicaid Services is doing an analysis, said Ms. Rucker, to determine whether the $600 threshold for specialty drugs should be changed.

Driving value requires a systematic health care team effort, along side patients and caregivers. "It takes a village, if you will, to drive value across the system, with patients, caregivers, and all stakeholders," stated Ms. Rucker. OptumRx specialty pharmacy conducted a study that identified opportunities for medication-related consults to improve value in multiple sclerosis. This study found that medication gaps accounted for 6%, and nonadherence was only related to 11% of the opportunity. Drug safety concerns accounted for 34%, and cost savings the remaining 49%. Drug adherence, safety, and therapy gaps may all be addressed through medication therapy management. However, this is truly a shared responsibility, she pointed out. For example, many medications are approved today with risk evaluation and mitigation strategies, which by and large, mandate the distribution of medication guides to patients. Yet, these guides commonly assume a fourth-grade reading level and the ability to navigate the health system, neither of which may be the case.

She believes that the trend toward self-administration of specialty drugs means less opportunity for patient counseling and face-to-face support with a professional. "This goes against the appeal

of high-touch engagement, which is an important way to motivate adherence and to deal with drug safety concerns," said Ms. Rucker.

LOOKING AHEAD TO ENHANCE VALUE

One ongoing project seeks to maintain the "high-touch" patients receive in the hospital even after they are discharged. Project RED (Reengineered Hospital Discharge), uses a computer program to engage the patient in a conversation about their health status, with a Web-based avatar, in the privacy of their own home. If the patient gives a response that is flagged, the program automatically contacts a practicing clinician to speak live with the patient.

Ms. Rucker indicated that there numerous opportunities to enhance value, and suggested that targeted medication therapy management for those utilizing specialty-tier medications may be a good place to focus. Patients can receive incentives to participate (no incentives at present in Part D, however), and shared savings incentives are offered to the drug plan and accountable physician practice. In Part D plans, the value of care management services can be demonstrated by conducting research into the outcomes of Medicare beneficiaries using specialty-tier medications by subsidy status.

She reiterated that patients should be part of the therapeutic decision loop, by inviting their feedback but not overburdening them with too much indecipherable information. She would like to see incentives aligned to better link prescription access and value.

HOW CAN WE AVOID REACHING THE TIPPING POINT? AN EXPERT PANEL DISCUSSION

A report on a discussion with panelists A. Mark Fendrick, MD, Director of the Center for Value-Based Insurance Design; John Rother, JD, President and CEO, National Coalition on Health Care; N. Lee Rucker, MSPH; Principal and Founder, Enhance Value; and Nadir Halim, PhD, MPH, MBA, Director, US Policy, Pfizer Inc.

PRACTICE GUIDELINE USE IN SPECIALTY GROUPS: A CARROT AND A STICK

An audience member asked the panel how they could address practice guidelines from specialty and professional groups that do not include any financial or cost considerations? From a VBID perspective, are these usable?

Mr. Rother responded, "Value is now the key to health policy and health practice. Guidelines that do not refer to value are not going to be helpful or relevant going forward." Dr. Fendrick said that politically, discussions do not happen surrounding costs. Most provider groups do not want to discuss denying care on the basis of cost, as this is considered "almost toxic." Organizations like AMCP and academic groups do want to address costs, "but are literally swimming against the current." Mr. Rother agreed that it is very hard to do from a public policy point of view. Instead, private provider organizations must take the initiative and begin to consider value.

Ms. Rucker believes that other professional societies, the American Society of Clinical Oncology in particular, are now considering cost. Dr. Halim emphasized that the evidence base should be first and foremost in developing these guidelines, with cost information second. In any case, said Ms. Rucker, "It's too late to consider value when the patient gets to the pharmacy and finds out what his or her cost share is."

The audience broadly agreed that if the clinical evidence supports the use of different options, the guidelines should include cost-effectiveness information on those products. "It is an inherent part of value," said one medical director.

In terms of optimizing value, step therapy can be a very useful tool. However, consumers have always viewed step therapy as a barrier to access, not as an assurance of quality or value, according to Dr. Fendrick. If the practice guidelines call for a patient to try one treatment before attempting another, he stated, how do we convince the patient that step therapy is actually a good thing, when we try to make it harder for patients to receive treatments that work better?

CALCULATING VALUE AT THE HEALTH PLAN LEVEL

Determining the relative value of one treatment versus another can be daunting. One attendee asked, "Where do you begin?" A pharmacy director in the audience agreed that "doing a value assessment for every drug is a major task." He offered one useful starting point: identifying the top 25 drug classes by volume and focusing on the high-cost drugs. His organization performed class reviews based on the evidence available to date, in an effort to stratify agents that provided

the highest value. Agents that did not distinguish themselves in this review simply remained in their original formulary position. For example, etanercept and adalimumab were placed on tier 2, but other lower-cost agents remained on tier 4, based on whether the evidence was strong enough that they had high value.

DIRECT-TO-CONSUMER ADVERTISING

With the concern over the specialty drug trend, it can be discouraging to see so many consumer-directed commercials for high-cost drugs. Patients seem to visit their physicians' offices, asking for the drug they saw on television, regardless of whether it is appropriate for them.

"It's too late to consider value when the patient gets to the pharmacy and finds out what his or her cost share is."

If the patient wants that direct-to-consumer (DTC)-advertised agent, suggested Dr. Fendrick, the clinician may be well served to inform the patient what the level of cost sharing will be, particularly if the drug is on a specialty tier.

Mr. Rother agreed that DTC advertising does drive consumption. "Is this the right way to inform the public? I have my doubts. Low-value products tend to get advertised," he said.

Ms. Rucker cautioned that the opportunity to self-serve, even in the prescription world, is growing, through illicit or noncertified Web-based businesses. The emphasis really needs to be on the physician who, when visited by a patient wanting a drug advertised on TV, can "whip out his or her trusty algorithm, explaining that we're moving to clinical pathways based on value. To the extent that low-cost options may clinically benefit you, we're not going to go with what you just saw on TV."

Dr. Fendrick added, "Better than DTC is over-the-counter (OTC), which is more germane to value." Although he understands the need to encourage the conversion of medications to OTC status because of unit price, he wondered whether it might be best to have the pharmacy adjudicate those OTC transactions as well, so they are tracked and included in the medical record. "We don't know whether my favorite medicine—aspirin—is taken enough."

OFF-LABEL USE OF SPECIALTY DRUGS

Specialty pharmaceuticals, like conventional medications, are often tried for indications other than what is listed on the approved label. However, when they are used off-label, the risk of wasted resources is greater, related to their higher cost. In many cases, managed care pharmacy departments cannot determine from the claims database *why* a drug was prescribed, only that it *was* prescribed. Dr. Fendrick was frustrated over this fact, and believed it to be unreasonable with today's data capabilities and the electronic medical record (EMR). Including the indication for prescribing the drug, he believes, would improve quality.

"This comes back to clinical nuance," he explained. "Organizations should value pharmaceutical agents differently for different indications (approved or not), and individuals should pay less for a really good on-label use compared with another on-label use."

DISRUPTIVE TECHNOLOGIES AND SPECIALTY PHARMACY

Managed care is seeking ways to deflect the specialty pharmacy cost trend and is hoping that the use of technology can stem the tide. Dr. Halim believes that the EMR and data mining can help in this respect. It gives us the ability to gather large amounts of data and change clinical practice. It gives us insight into "big data." Dr. Halim said, "In the drug development world, there are lots of

advances in translational sciences, [and] in basic research that has a huge potential to impact the development of drugs, with major consequences for the cost of drugs. Mr. Rother added that postmarketing surveillance registries can also have a great effect, even in tracking patients who should be taking their medications but don't.

THE ROLE OF PATIENT-REPORTED OUTCOMES

Postmarketing surveillance or disease-specific patient registries offer a broad opportunity to track patient-reported outcomes (PROs). The reporting and use of PROs are in their very earliest stages today but have the potential to transform several aspects of care, including drug development. Ms. Rucker believes that PROs will be valuable, but "it might take a bit of a transition to get patients to take part in the process. For so many years, patients have not been asked how the drug was working. We have to first have generic conversations with them about how they matter to the health care team. Patients need to be invited and encouraged." As we see more measures related to functional status, registries will help drive their use, she added.

"How do we convince the patient that step therapy is actually a good thing, when we try to make it harder for patients to receive treatments that work better?"

Dr. Halim commented that "it really is important to figure out how to incorporate PROs into drug trials. It could help quicken the development of the next generation of drugs." He illustrated how the development of drugs for Alzheimer's disease or other mental health disorders will rely on direct feedback from patients, in addition to clinical markers. He also pointed out that PROs are very important to identifying and addressing areas of unmet medical need.

Once we commit to prescribing an $84,000 medication, Dr. Fendrick said, we also need to emphasize the role of personal accountability. We need to put some of the onus on the consumer, and empower them. He explained that preliminary data on patients taking Sovaldi show that 1 in 12 did not complete their medication course. If they only take a portion of it, tens of thousands of dollars are wasted. It may be that the patient must commit to adherence monitoring of some sort as part of the agreement to received treatment with this drug.

INCREMENTAL IMPROVEMENT, VALUE, AND THE TIPPING POINT

Some oncology therapies have been shown to improve survival only 1 to 2 months or improve progression-free survival but not overall survival. This does not seem helpful in the effort to avoid going over the specialty pharmacy tipping point. Attendees asked for advice in assessing the value of these treatments in this context.

Dr. Halim acknowledged that in randomized clinical trials of oncology agents, progression-free survival or overall survival are often the primary endpoints being measured. "Oftentimes, with oncology treatments, we don't see the full benefit or value of the product until we get years of patient experience postlaunch. We need to be a little cautious," he said, "about focusing our value discussions on just those endpoints from clinical trials. This doesn't mean that it is all the drug provides. For example, in breast cancer, a recent study found increases in survival well beyond what the initial investigational studies showed." Although, as Dr. Halim said, value of a medication can improve over time, Mr. Rother pointed out that it can also deteriorate over time. If value does increase over time, perhaps the drug price should be allowed to rise, and if the value decreases, the price should drop as well. "We have to be more creative in addressing

this down the road, and between who we are asking to pay and who is receiving the benefit," he said.

Up to 80% of oncology prescribing is off-label, said Ms. Rucker, and this "goes to the heart of the patient trusting their doctors. Also, among the very elderly, there may be personal or clinical reasons why patients would want to avoid interventions that prolong life [by just] a couple of months." Mr. Rother agreed that care at the end of life is a very difficult area: "We should be very careful about offering expensive drugs at the very end. It's not anyone's definition of value. We should be careful about promoting it."

THE BROADER IMPLICATIONS

If we consider the example of caring for premature births, our society is very willing to invest well over $1 million per child, "where it lowers or prevents incremental costs throughout the life of the infant," said Dr. Fendrick. "It's not that we're not willing to make the investments up front. It's the magnitude and volume of the Sovaldi issue that is problematic—not the individual cost of it."

"When thinking about creative reform from a policy perspective, we need to stop carving out drugs and align incentives to encourage appropriate prescribing."

Mr. Rucker thought it might be possible to recalibrate our estimates of value if payers applied multiyear rather than annual budgeting. Perhaps that would require an incentive for members to stay within the same plan.

WHEN WILL WE REACH THE TIPPING POINT?

Dr. Halim reminded the attendees that drug spending accounts for only about 10% of the total health care expenditure. Drug advances have addressed unmet needs, and new specialty pharmaceuticals have provided treatments where none had existed before. Perhaps bringing out more specialty pharmaceuticals, not less, is a societal good.

Ms. Rucker pointed out that if drugs continue to be carved out of the bundled payment, even in accountable care organizations, it will be difficult to make a value case for them based on their impact on other aspects of health care. "When thinking about creative reform from a policy perspective, we need to stop carving out drugs and align incentives to encourage appropriate prescribing," she asserted.

The temperature is rising "but not boiling yet," said Mr. Rother. Health care costs have been rising more slowly than originally predicted, and perhaps the increasing specialty pharmacy spend has had an influence on this trend. Also, the fact that several conventional medications have gone off patent may have played a role. Regardless, he explained, the slower rise in health care spending won't last—Medicare costs are rising with the aging of the baby boomers. "A year ago, I could not have even thought of having this conversation. We're actually having this conversation today because one company went too far in pricing one product that treats too many people, even though it's a good product," stated Mr. Rother.

"That's what triggers these changes in attitudes—not all at once but gradually. The tipping point will be economic. When we can't do other things considered essential, and we're close now, because of all the money we're throwing at health care, then it's a new ballgame," he concluded.

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