Jeff M. Myers
President and CEO

Medicaid Health Plans of America
Ensuring Access to Better Healthcare

March 4, 2016

The Honorable Ron Wyden
United States Senate
Washington, DC 20510-

Dear Senator Wyden:

Thank you for providing an opportunity to comment on "The Price of Sovaldi® and Its Impact on the U.S. Health Care System" released in December 2015.

Medicaid Health Plans of America (MHPA) is the leading national trade association representing private sector Medicaid managed care plans, ranging from multi-state, for-profit plans to small, non-profit plans. MHPA's 127 health plan members serve over 28 million Medicaid enrollees in 34 states and D.C. In 2015, private sector managed care plans covered 70% of the total Medicaid enrollees, making our members important stewards of our nation's publically funded safety net programs.

As suggested in your report, public sector health care programs like Medicaid are disproportionately impacted by the exorbitant pricing structure of Sovaldi® and Harvoni®. The trade association and our member companies noted with great interest the findings of the report that clearly indicate that Gilead sought to capture projected savings up front (pages 40-42). This is the current pricing model that pharmaceutical companies utilize and which is unsustainable in the long-term. It simply is not feasible in today's dynamic environment to expect to seize all of the future "savings" today as a cost. Our trade association is committed to working with life sciences manufacturers to find a better way for innovators to receive value for their discoveries, AND for society to receive value in the utilization of those new products.

We would also like to point out that it is not unusual for the documented outcomes of highly controlled clinical trials (required for FDA approval) to differ substantially from what actually occurs after the drug is released for use in broader population. It is after a newly approved drug is released for general use that the true efficacy and scope of adverse events become more clear, and this cost/value equation directly impacts the plans that are at risk and provide care for our nation's sickest and poorest. As you continue to seek solutions to the impact of exorbitantly priced innovator medications on public safety net programs, MHPA encourages you to consider requiring pharmaceutical manufacturers

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share data on post launch efficacy and outcomes to assist in determining the true value of the medication long term.

From our point of view, one of this issues Congress should consider is the way in which Section 1927 of the Social Security Act impacts both life sciences companies and the Medicaid program overall. Section 1927 establishes the best price/rebate/guaranteed access provisions specific to the drug benefit in the Medicaid program. As provided under the statute, a pharmaceutical manufacturer wishing to take advantage of guaranteed coverage of their products through Medicaid need only enter into an agreement with the Secretary for providing a rebate - currently 23.1%, or the best price in the commercial market. In the case of Sovaldi® and the Committee's report, the manufacturer essentially raised the price to cover the rebate knowing that it would be very challenging to reduce or control demand. Our Association is concerned about the impact of this section on the overall program, which directly impacts enrollee health, and will be working with interested parties to provide different options to you and other policy makers to consider. Recently, Eli Lilly, a major pharmaceutical company, and Anthem, a large Medicaid insurer and an MHPA member, produced some policy "think pieces" that may guide this discussion. We have included their white papers with this letter for your information and would welcome any thoughts you may have on them.

Something must be done to encourage more risk sharing and a move to a new model of payment for life sciences innovations. While your report focused only on Gilead's release of its Hepatitis C medications and the price impacts, the truth is that the overall life science marketplace – both innovator and generic -- is moving to a "raise the price because we can" model.

It is striking, and unsustainable, to see an important segment of the health care ecosystem increase prices in the following manner:

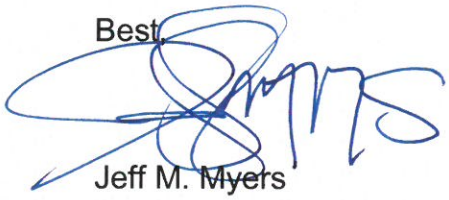
- Of 3000 Branded Drugs:
  - 1/2 increased YoY;
  - 1/3 <10% YoY;
  - 60 <100%
  - 20 <400% or more (Bloomberg; "Shkreli Was Right, Everyone's Hiking Drug Prices" 2-2-16, research by DRX)

Over the next several months, MHPA is undertaking a policy development discussion among our members to identify recommendations on how to improve the current structure. Our discussion will explore payment options (and possible changes to the structure of 1927) that focus on high quality care, good outcomes, and effective management of resources rather than on an artificially set pricing floor created by mandatory rebates.

March 4, 2016

Again, thank you for the opportunity to participate in this important discussion. If you have additional questions, please contact me or Jeannine M. Bender, Senior Director Federal Relations at 202-857-5729; jbender@mhpa.org.

Best,



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While the rest of the healthcare system is paying for value, payments for drugs largely continue to be stuck in a 20th century construct that focuses on price, regardless of the health outcomes of each patient. Anthem and Eli Lilly and Company are collaborating to help accelerate the transition towards a value-based system with policy proposals that will help drive payment innovation. Read more at ThinkAnthem and LillyPad.

Promoting Value-Based Contracting Arrangements

Eli Lilly and Company and Anthem | January 29, 2016

The following memorandum outlines Eli Lilly and Company and Anthem's joint perspective on creating legislative and regulatory options designed to promote value-based contracting arrangements for manufacturers and commercial health plans.

POLICY GOAL: Create a policy environment conducive to allowing health plans and manufacturers to enter into a variety of value-based contracting arrangements, aligned with the shift toward value-based payment and the goal of promoting access to high-value care. This may include creation of legislative/regulatory exceptions for Best Price and all other relevant government pricing calculations and requirements as they relate to products sold or transferred under value-based contracts, as well as additional safe harbors to the federal Antikickback Statute (AKS) that protect value-based contracts from AKS liability.

Background and Context

Policymakers and industry are looking for opportunities to drive quality, create savings, and slow cost growth in the healthcare system. In particular, stakeholders are increasingly focused on value and increasing the use of treatments, products, and services that are both clinically effective and efficient. In fact, by 2018 the Department of Health & Human Services intends to link 80 percent of Medicare payments to value, and 50 percent to alternative payment models like bundled payments.[1]

While there has been substantial innovation in how health plans and the government reimburse for hospital and physician payments (with physician payments moving further in this direction following the recent Medicare and Children's Health Insurance Program (CHIP) Reauthorization Act), payment for prescription drugs is often based on more traditional outcomes (e.g., volume of product purchased). Given the role that prescription drugs play in the treatment of and spending for many complex and chronic conditions, health plans and manufacturers seek greater opportunity to align payment with quality, accountability, and coordination. Ultimately, these arrangements can encourage access to high-value medicines and treatments and ensure that patients are getting the best value for their healthcare dollars. However, existing legal and regulatory barriers are stifling this innovation.

Traditional drug pricing contracts typically establish a fixed price for the product that remains in place throughout the benefit year. This may include flat pricing (e.g., price per unit) or volume arrangements. For example, a health plan and manufacturer may agree that if spending for a particular medicine exceeds a certain level, the manufacturer will offer the health plan a discount or rebate on the unit price. In contrast, value-based arrangements may condition payment or drug price based on patient-level clinical or economic outcomes (e.g., a 1 percent reduction in HbA1c levels for certain patients with diabetes). As illustrated in Appendix A, these arrangements represent a fundamental departure from traditional contracting and may require investment from both manufacturers and health plans. In order to design a contract that makes payment for a therapy contingent on patient outcomes, for example, the manufacturer and the health plan must agree on how to reliably, accurately, and appropriately measure outcomes and categorize patients as "responders" or "non-responders." In addition, both plans and manufacturers need to designate staff to monitor and execute the agreement, a further investment by both parties.

[1] HHS.gov. "Better, Smarter, Healthier: In historic announcement, HHS sets clear goals and timeline for shifting Medicare reimbursements from volume to value." 26 January 2015. Available at: http://www.hhs.gov/news/press/2015pres/01/20150126a.html

Value-based arrangements allow both parties to share in the risk associated with individual patient outcomes and spending for a particular medicine. These contracts offer important benefits for consumers, health plans, and manufacturers. Consumers, in particular, may enjoy earlier or less managed access to new medicines when payments are aligned with value. Over time, identifying and encouraging use of the highest-value products could both improve patient outcomes and curb healthcare spending growth. Although manufacturers and health plans seek opportunities to invest in these innovative models, existing legislation and regulations raise concerns, including:

- **Anti-Kickback Statutes:** Federal and state fraud and abuse laws are designed to protect patients, health plans, and the healthcare system overall from fraud, waste, and abuse. The Anti-Kickback Statute (AKS) prohibits offering or receiving remuneration (broadly defined) to induce or reward referrals for items or services paid for by federal healthcare programs. Statutory and regulatory safe harbors protect certain arrangements from AKS liability, but it is unclear how enforcement agencies would apply these safe harbors to value-based arrangements.[2] AKS violations carry significant financial and other penalties.
- **Government pricing:** Manufacturers are required to report pricing data to the federal government to determine Medicaid rebates; Medicare Part B payment rates; the 340B program ceiling price; and the maximum price that certain government agencies can be charged. Because these reporting requirements did not foresee and were not designed to be compatible with value-based contracting, they could make it exceptionally difficult for a manufacturer to enter into a value-based contract. For example, current Medicaid rebate regulations would require that rebates paid to a commercial health plan in the context of a single value-based contract be made available to Medicaid programs, even though Medicaid programs would not be subject to the key design features of the value-based arrangement (for high level examples of value-based arrangements, please see Appendix A).

In order to capitalize on the potential of value-based arrangements for consumers, industry, and other healthcare stakeholders, policymakers should adapt standards related to kickbacks and government pricing to accommodate these novel contracts. Absent such clarification, legislation and regulations that reflect traditional contracting will continue to impede innovation.

Promoting Value-Based Arrangements

The goals of value-based contracting approaches align with the shift toward high-value care. Such agreements seek to make more efficient use of healthcare resources, potentially reducing costs throughout the system, encouraging use of the most efficient and effective care, and improving patient outcomes.

Clearer safe harbors and guidance for all stakeholders could promote the adoption of value-based models. For example, stakeholders could benefit from:

- Expansion or creation of new statutory or regulatory safe harbors, including by the OIG, to the AKS that more clearly protect innovative, value-based contracting arrangements (under a defined set of requirements);
- Amendments to the statutory and regulatory definitions of certain prices reported to the government (e.g. Best Price) to define the terms for excluding payments made pursuant to value-based contracts;
- Regulations clarifying how manufacturers can incorporate value-based contracts in their price reporting calculations; and,
- Pilots or demonstration programs with commercial health plans that adapt the government pricing rules to test how value-based contracts impact reported government price figures, and in turn, Medicaid rebate amounts and 340B ceiling prices.

Existing Legislative and Operational Barriers

FRAUD AND ABUSE

As described above, current fraud and abuse guidance may have a chilling effect on value-based arrangements. For example, if a manufacturer and health plan entered into an arrangement under which the manufacturer accepted risk for treatment failure (e.g., the health plan would only pay for a drug for patients with positive treatment outcomes), this could be viewed as remuneration offered to encourage the health plan to favorably cover the manufacturer's product. Additionally, the OIG has not issued sub-regulatory guidance addressing value-based contracting arrangements, leaving stakeholders with little to no insight on OIG's position on the risk of fraud and abuse they pose.

To alleviate stakeholder concerns related to fraud and abuse, regulations and guidance could be updated to establish regulatory[3] safe harbors, and the OIG could update guidance[4] in order to explicitly protect these arrangements when certain criteria are met. Congress could also consider establishing a statutory safe harbor. The safe harbor eligibility criteria can be designed to minimize risk of fraud and abuse in these arrangements. For example, the OIG could require that value-based payments be determined in advance, and could not vary during the term of a contract.

[3] 42 C.F.R. § 1001.952
[4] 66 Fed. Reg. 23731 (May 5, 2003)

GOVERNMENT PRICING

Manufacturers' government price reporting obligations may also have a chilling effect on the development of value-based contracts. Largely, this is a result of the way pricing, discounts, and contracting arrangements affect government price reporting, and in turn, manufacturer obligations under a variety of federal programs.

Best Price and Average Manufacturer Price

Implications for Best Price are the most immediate, important factor to consider with respect to manufacturer incentives created by value-based contracting. Best Price is the pricing benchmark Medicaid uses to ensure state Medicaid programs never pay more than the lowest price offered for a particular therapy. Best price is set based on the single "lowest price available from the manufacturer during the rebate period to any entity[5] in the United States."[6] Best Price is affected by manufacturer rebates, discounts, or other price concessions to commercial health plans, and setting a new Best Price can lead to significantly increased Medicaid rebate and 340B program liability for manufacturers. Under the current regulations, payments from manufacturers to health plans under a value-based contract would almost certainly need to be included in Best Price calculations. For example, if a manufacturer offered a health plan a rebate equal to 60 percent of the list price of a product for each enrollee who did not respond to treatment, and negotiated a payment rebate equal to 20 percent of the list price for patients who did respond, the 60 percent rebate would determine the product's Best Price for all patients. If the list price were $1,000, this would result in a Best Price of $400 (assuming that there were no greater discounts/rebates in the market; if so, Best Price would be even lower). As the example below shows, reductions in Best Price can result in significant Medicaid rebate and 340B liability.

	List Price	AMP[7]	Best Price (BP)	Medicaid Rebate Amount per Unit (AMP-BP)*[8]	340B Ceiling Price (AMP – Medicaid Rebate Amount)
Without value-based rebate (example)	$1,000	$950	$700	$250	$700
With value-based rebate (including patients who do not respond to therapy)	$1,000	$950	$400	$550	$400

* Assumes no additional "inflation-based" rebate

These reductions may create negative returns on value-based contracts for manufacturers. The Best Price statute and regulations could be updated to exclude from Best Price certain rebates and other price concessions paid from manufacturers to health plans that are the result of value-based contracting (e.g., additional rebates paid if a patient does not respond to treatment). CMS could also be authorized to create pilot programs to test how value-based contracts and alternative approaches to Best Price impact reported government price figures, and in turn, Medicaid rebate amounts and 340B ceiling prices.

Further complicating compliance with the price reporting rules is the fact that many value-based arrangements could constitute "bundled arrangements," which require complicated re-allocation of price concessions, either across products or across periods, and trigger additional disclosure obligations.

Average Sales Price

Best Price is not the only price point that would be impacted by value-based contracts. For medical benefit drugs (in general, those that are physician-administered), manufacturers are required to report Average Sales Price (ASP) to the Centers for Medicare & Medicaid Services (CMS) on a quarterly basis; ASP is used to set Medicare Part B reimbursement rates.[9] ASP is defined as "the manufacturer's sales to all purchasers…in the United States for a drug or biological in the calendar quarter [excluding exempted sales] divided by…the total number of such units of such drug or biological sold by the manufacturer in such quarter."[10] Like Best Price, rebates to commercial health plans are included in ASP, but unlike Best Price, ASP is an average price and is not determined by a single sale.[11]

Manufacturers of medical benefit products may be concerned that substantial rebates offered to commercial health plans under value-based contracts would reduce ASP, and in turn, Medicare Part B reimbursement rates for their products. Relatedly, CMS may substitute Average Manufacturer Price, or AMP, for ASP under certain conditions. AMP is also used in determining Medicaid rebates, and a product's

[5] With certain exceptions, including most government purchasers and Part D plans.
[6] 42 C.F.R. § 447.405
[7] Assumes AMP = 95% of List Price.
[8] As BP is used in this particular example, it is assumed that this price (AMP-BP) is greater than statutory requirements in this particular scenario.
[9] 42 U.S.C § 1395w–3a(b)(1).
[10] 42 U.S.C § 1395w–3a(c)(1).
[11] 42 U.S.C § 1395w–3a(c)(2). The ASP statute references the statutory definition of Best Price in defining what rebates and discounts are excluded from ASP; in other words, the same rebates and discounts that are excluded from Best Price are also excluded from ASP. Therefore, changes to the definition of Best Price would also impact manufacturer's ASP calculations.

340B ceiling price. Accordingly, it will be critical for policymakers to address the implications of value-based arrangements for government prices in a consistent and comprehensive manner.[12]

OPERATIONAL COMPLEXITY

Value-based arrangements are complex, and manufacturers and health plans will need to align expectations with respect to desired outcomes, data collection, appropriate safeguards for patient privacy, and other factors. Manufacturers and payers are willing to work through these challenges, but the up-front investments necessary to make value-based arrangements succeed underscore the importance of appropriate regulatory and financial protection.

Manufacturer reporting and operational responsibilities under current pricing rules also pose a challenge. Accurately calculating and reporting government pricing figures are extremely complex tasks. Many pharmaceutical manufacturers expend significant resources on these activities. Value-based arrangements, in which the ultimate "price" of a product may not be known for a substantial period of time after the product is sold, would introduce significant operational complexity into these calculations and potential for legal ramifications. Additionally, many innovative contracting arrangements do not price products on a per-unit basis; for example, a course of therapy or treatment regimen price may be used instead. Incorporating non-unit based pricing into government pricing calculations may be very complex.

Key Considerations for Implementation

Several strategic considerations are critical to ensure the success of value-based contracts under a new regulatory paradigm. First, value-based contracts must be properly defined through federal regulation in order to accommodate existing and future goals. The definition will need to establish whether a value-based arrangement must have certain core features (e.g., risk sharing) in order to be eligible for any legislative and regulatory carve-outs, and, if so, what those core features are. While defining such an arrangement is necessary to ensure stakeholders have a common understanding of the arrangement, the definition will need to be sufficiently flexible to allow for potential innovative structures that have yet to be developed.

The government should simultaneously assess how best to update relevant laws and regulations in order to accommodate these innovations. For example, regulators could develop a template memorandum of understanding for payers and manufacturers to use, or a sample agreement between a manufacturer and the government that would appropriately exclude value-based pricing concessions from reporting. In addition to updating directly relevant laws and regulations (e.g., those related to pricing), regulators will need to consider whether existing fraud, abuse, and monitoring paradigms protect all stakeholders – including patients – impacted by value-based arrangements.

Finally, it will be important to allow for the emergence of value-based arrangements as part of the broader health system transformation and assure continued efficiency for government programs and patient access. By creating an environment in which value-based contracts are permitted, increased collaboration between health plans and manufacturers to enter into value-based contracts presents a significant opportunity to drive quality and access, create savings, and slow cost growth, aligned with the value-based shift in the overall US healthcare system.

[12] It is important to note that AMP for 5i products, which are infused, instilled, implanted, injected, or inhaled products that are generally not dispensed through a retail community pharmacy, includes additional rebates. Further, if a particular product is not sent through a distributor for 90% of gross sales, then the Non-Federal Average Manufacturer Price (NFAMP) is affected as payer discounts are factored in due to the 90-10 rule.

Appendix A

The below graphic depicts the level of risk on behalf of the health plan and manufacturer as well as the degree of difficulty of implementation of value-based contracting arrangements.

	Traditional Discounting	Conditional Payment	Value-Based Arrangements
General Description	Drug price is established prior to coverage and fixed for the benefit year	Payment contingent on certain short-term health outcome or evidence collection target	Reimbursement is tied to clinical or process outcome at the individual patient level
Key Inputs	Negotiated discount or rebate	Pre-determined goal for a defined patient population (e.g., short-term treatment goal such as persistence)	Pre-determined goal for a defined patient population (e.g., 1% reduction in HbA1c, performance versus competitor, delay in disease progression)
Key Outcomes	Varies (e.g., flat pricing, volume of drug purchased)	Attainment of treatment goals or collection of additional evidence through research	Patient-level clinical or process outcome (may occur after benefit year ends)
Example	• Market share-based rebating or price-volume arrangements • Utilization cap or manufacturer-funded treatment initiation	Coverage/payment with evidence development or conditional treatment continuation	Manufacturer provides rebate on products purchased for patients who fail to achieve desired outcome seriously consider aggressive utilization management criteria and stakeholders scramble for mid-year solutions.

Degree of Difficulty and Risk *The specifics of any type of value-based arrangement vary, and are negotiated by the plan and the manufacturer*

Source: J Carlson, et al. "Linking payment to health outcomes: A taxonomy and examination of performance-based reimbursement schemes between healthcare health plans and manufacturers." Health Policy. 2010 Aug;96(3):179-90.

Appendix B

The following tables outline the statutes and regulations by topic area as relevant for this memorandum.

Fraud & Abuse	
Legislative or Regulatory Change Desired	**Corresponding Statute/Regulation**
Addition of statutory safe-harbor to existing language	42 U.S.C. § 1320a-7b
Addition of regulatory safe harbors to existing language	42 C.F.R. § 1001.952
OIG guidance to pharmaceutical manufacturers should be updated	66 Fed. Reg. 23731 (May 5, 2003)

Government Pricing	
Definition Referenced	**Corresponding Statute/Regulation**
Statutory definition of Best Price	42 U.S.C. § 1396r–8
Regulatory definition of Best Price	42 C.F.R. § 447.505
Statutory definition of ASP[13]	42 U.S.C § 1395w–3a
Regulatory definition of ASP[14]	42 C.F.R. § 414.804

[13] Note that if the statutory definition of BP is updated to exclude value-based payments, they will also be excluded from the statutory definition of ASP due to the fact that the ASP statute cross-references the BP statute
[14] Note that if the statutory definition of BP is updated to exclude value-based payments, they will also be excluded from the regulatory definition of ASP due to the fact that the ASP regulation cross-references the BP statute

While the rest of the healthcare system is paying for value, payments for drugs largely continue to be stuck in a 20th century construct that focuses on price, regardless of the health outcomes of each patient. Anthem and Eli Lilly and Company are collaborating to help accelerate the transition towards a value-based system with policy proposals that will help drive payment innovation. Read more at ThinkAnthem and LillyPad.

Facilitating Open Communication About Emerging Therapies

Eli Lilly and Company and Anthem | January 29, 2016

The following memorandum outlines Eli Lilly and Company and Anthem's joint perspective on creating a legislative or regulatory exemption that would allow manufacturers and health plans to communicate about emerging products prior to U.S. Food & Drug Administration (FDA) approval.

POLICY GOAL: Clarify federal law to confirm that manufacturers may speak openly with health plans about drugs going through the FDA approval process, particularly with regard to product efficacy, safety, and pharmacoeconomic information.

Background and Context

Aligning drug coverage decisions with rate development and budgeting timelines – that is, making a new drug available within an insurance premium that reflects expected spending on that drug, among many other factors – requires thoughtful synchronization over a several-month period. Two recent developments have created an environment where this alignment is difficult for health plans[1], state and federal governments, and manufacturers to achieve, ultimately creating downstream consequences for consumers.

First, with changes to commercial insurance processes included in the Affordable Care Act, insurance rates in most lines of business (including the individual market, employer market, Medicaid, and Medicare) are set – and unchangeable – well before the coverage year begins (see Appendix A). Accurate rate development relies on detailed assumptions about the use of healthcare services, including prescription drugs. Manufacturers are the best source of reliable and accurate clinical and pharmacoeconomic information about emerging therapies, but face significant restrictions on communicating to plans information about not-yet-approved products. When plans cannot accurately account for a drug when setting rates, they may overestimate the drug's cost, setting premiums higher than is ultimately needed to cover the costs of the drug, and perhaps discouraging some consumers from purchasing coverage. Alternatively, plans might underestimate the cost of the drug, thus setting premiums that do not cover costs. As a result, plans may need to consider mid-year solutions to control budgets like more stringent utilization management criteria.

At the same time, the new U.S. Food & Drug Administration (FDA) "breakthrough" therapy designation process means that blockbuster drugs will likely be moving more efficiently through the development, review, and approval processes. While more rapid approval of breakthrough medicines means that these products are available to consumers sooner, health plans, employers, and state and federal governments have less time to plan for their utilization. "Breakthrough" drugs by definition offer significant benefits for consumers – the designation is granted when preliminary clinical evidence shows they may represent substantial improvement over available therapies for treatment of a serious condition – but can have significant budget impacts on health plans, employers, and state and federal governments. Given current health plan rate development and budgeting requirements, some of the most promising new medicines are most vulnerable to timing challenges and their potential implications for patient access.

Clarifying that manufacturers and health plans may discuss critical scientific evidence and other drug features (e.g., dosage, pharmacoeconomic information, and clinical indications) for products under FDA review would encourage these discussions, allowing health plans to better anticipate effects on the target patient population and consider available pharmacoeconomic and efficacy data. Stakeholders could design the clarification to preserve FDA's interests, as discussed below.

[1]While this document references health plans throughout, the policy solution would also aid other stakeholders including pharmacy benefit managers and certain providers who purchase drugs.

Existing law and regulations are intended to achieve a very specific and important goal: preventing the marketing of a drug before FDA determines that it is safe and effective for its intended use. However, the FDA regulations on this subject, namely 21 C.F.R. § 312.7, are broadly written and could be interpreted to sweep in beneficial and otherwise non-harmful communications. Health plans have long used sophisticated methods for assessing the evidence base and patient access considerations for new medicines, and are well positioned to assess manufacturer-generated evidence and consider any existing limitations in evidence available prior to FDA approval. However, given the lack of clarity on the boundaries of permissible, pre-approval scientific exchange, manufacturers are hesitant to discuss clinical data or pharmacoeconomic information until FDA approves a product.

While pre-approval communication between manufacturers and health plans should explicitly be lawful and permissible, policy safeguards could prevent the distribution of pre-approval information to other audiences, such as patients, who may not be equipped to interpret it. For example, manufacturers and health plans could enter into appropriate non-disclosure agreements. As is the case today, a therapy would not be used prior to FDA approval under the proposed solution, and health plans would still have ample opportunity to review the label post-approval. In addition, manufacturers might commit to follow up with health plans when necessary to correct any assumptions made in pre-approval discussions that materially differ from the final product labeling agreed with FDA.

Facilitating Open Communication

Removing the existing uncertainty about as to when and how manufacturers and health plans can communicate about certain critical clinical and safety aspects of a drug's attributes before FDA approval could benefit patients, health plans, state and federal governments, employers, and manufacturers. Patients could directly benefit in the form of less restricted access to new medicines and more predictable trends in insurance rates from year to year. Health plans would have additional, directly relevant information when establishing rates and benefits for the upcoming year. Manufacturers would have the opportunity to engage in more timely conversations with health plans, providing targeted and well-timed information that may be helpful when price negotiations occur.

The Unfortunate Lack of Communication: A Case Study

The Case

- Health Plan X is developing its rate for the upcoming year. Final rates are due in April.
- Health Plan X knows that breakthrough Drug Y is undergoing FDA review. Drug Y is expected to significantly improve outcomes and experience for patients with a certain type of cancer. However, Health Plan X does not know which patients will be eligible to take Drug Y. The manufacturer of Drug Y is unable to discuss Drug Y's target patient population or potential price with Health Plan X.

Options for Health Plan X	OPTION ONE: Health Plan X Overestimates Utilization of Drug Y	OPTION TWO: Health Plan X Underestimates Utilization of Drug Y
Health Plan X Pre-Approval Assumption	Health Plan X assumes that 3 percent of its members will take Drug Y during the year. Health Plan X also estimates price and factors these assumptions into rates and thus into the budgets of health plans, employers, state and federal governments.	Health Plan X assumes that 1 percent of its members will take Drug Y during the year. Health Plan X also estimates price and factors these assumptions into rates and thus into the budgets of health plans, employers, state and federal governments.
Drug Y is Approved	Drug Y is approved by the FDA in September. Based on the FDA-approved label, only 1 percent of Health Plan X's members are candidates for Drug Y.	Drug Y is approved by the FDA in September. Based on the FDA-approved label, 3 percent of Health Plan X's members are candidates for Drug Y.
End Result	Health Plan X overestimates the aggregate costs associated with Drug Y and develops rates that are higher than they would have been, had Health Plan X had access to more complete information. Fewer consumers purchase coverage because rates are higher than they should have been, and other areas of employer, state and federal budgets are potentially unnecessarily cut.	Health Plan X underestimates the aggregate costs associated with Drug Y and develops rates that do not adequately reflect actual costs. Health plan X and its customers (employers, state and federal governments) more seriously consider aggressive utilization management criteria and stakeholders scramble for mid-year solutions.

FDA's interests would be protected as well. Manufacturers would continue to have the same strong incentives to seek FDA approval, as commercialization would continue to be dependent on it. Further, new guidance could contain explicit safeguards to protect patient safety and to ensure that communications are truthful and non-misleading, and do not compromise FDA's interest in ensuring the safety and effectiveness of new medicines. For example, manufacturers could include prominent disclaimers on data shared with health plans, making it clear that the data are not FDA-approved. A more detailed analysis of potential stakeholder impacts is included in Appendix B.

Therefore, policymakers and regulators should clearly acknowledge through clarification of federal law and regulations that manufacturers may communicate with health plans about drugs undergoing FDA review, including potential indications, clinical performance and pharmacoeconomic information.

Existing Legislative and Operational Barriers

Current rules prohibit manufacturers from communicating promotional claims of a drug's safety or efficacy for an investigational use, and manufacturers must avoid commercialization of the drug before it is approved for commercial distribution.[2] FDA's chief concern is that such communication may undermine the approval process, as products' safety and efficacy are not considered to be established until FDA grants marketing authorization. Further, FDA is concerned that pre-approval communication could create confusion if stakeholders share safety and efficacy information that is not ultimately included in the FDA-approved label. Currently, an existing regulation provides that:

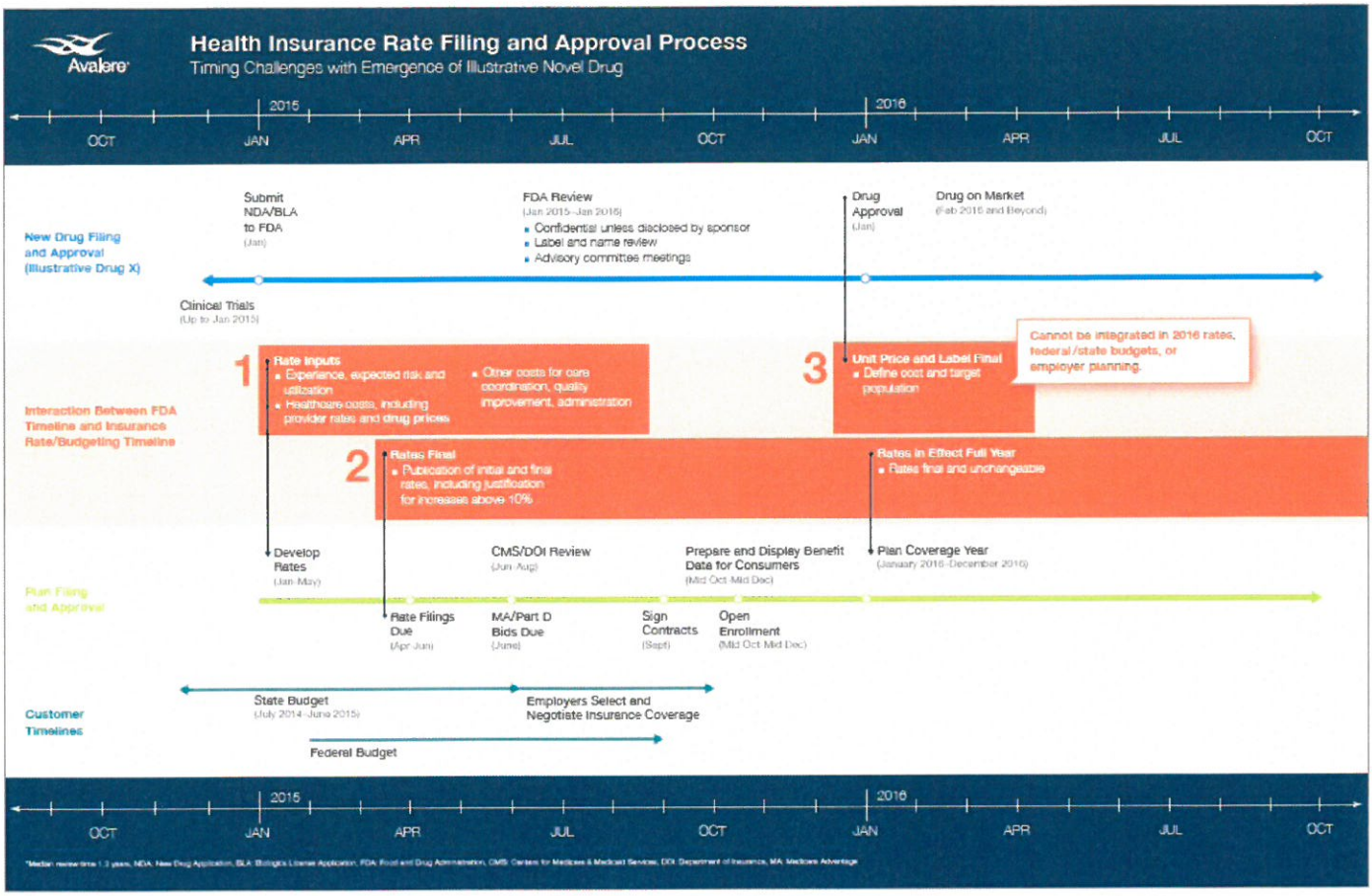
> *A sponsor or investigator, or any person acting on behalf of a sponsor or investigator, shall not represent in a promotional context that an investigational new drug is safe or effective for the purposes for which it is under investigation or otherwise promote the drug. This provision is not intended to restrict the full exchange of scientific information concerning the drug, including dissemination of scientific findings in scientific or lay media. Rather, its intent is to restrict promotional claims of safety or effectiveness of the drug for a use for which it is under investigation and to preclude commercialization of the drug before it is approved for commercial distribution.*[3]

However, targeted pre-approval conversations between manufacturers and health plans are not inherently promotional, and in the absence of definitive negotiations or a signed agreement, such conversations would also not amount to commercialization. In any event, use of a medical product would not be allowed prior to FDA approval. While such conversations are not clearly covered or prohibited by existing legislation or regulations, ongoing concerns about FDA's interpretation have a chilling effect on industry and create a compelling need for clarification.

Key Considerations for Implementation

Clarifying manufacturers' right and ability to communicate with health plans about certain pre-approval, product-specific information raises several practical considerations. First, key stakeholders (i.e., FDA, manufacturers, health plans) would need to define the specific types of information that are critical to inform desired discussions. We expect this information would include data from pivotal Phase III clinical trials (supported by substantial evidence) or pharmacoeconomic data (supported by competent and reliable evidence). Additionally, stakeholders should consider what limitations and parameters must guide these discussions (e.g., the discussions should be limited to indications undergoing FDA review, should be within a certain time period of the expected PDUFA date, should be subject to confidentiality restrictions, and should contain appropriate disclaimers). Manufacturers and health plans would need clear guidance from FDA on these topics, as well as compliance and oversight expectations.

FDA's existing regulatory framework for post-approval communications – that they be truthful and non-misleading – offers one potential path forward for setting expectations and guardrails for pre-approval discussions.[4] Applying a standard to pre-approval communications that is similar to the truthful and non-misleading standard applied to post-approval communications has several important benefits. Notably, this would ensure consistency over time (pre- and post-approval). Key stakeholders are already familiar with the standard, which would facilitate implementation of such an approach for pre-approval discussions. By applying a familiar but rigorous standard to pre-approval communications, while clarifying the parameters of these discussions, FDA can inject greater consistency into its regulatory framework and encourage richer discussions among stakeholders, ultimately benefitting patients and other stakeholders.

[2] 21 C.F.R. § 312.7
[3] 21 C.F.R. § 312.7
[4] See 21 U.S.C §§ 352(a), 321(n); 21 C.F.R. § 202.1(e)(3)(i).

Appendix. Health Insurance Rating Filing and Approval Process