

New Hepatitis C Treatments: Considerations and Potential Strategies for States

Executive Summary

The U.S. Food and Drug Administration recently approved three new drug therapies with the potential to cure a significant portion of the more than 3 million people in the United States estimated to be infected with the hepatitis C virus (HCV).

The three therapies—sofosbuvir (Sovaldi, Gilead Sciences); its successor, ledipasvir + sofosbuvir (Harvoni, Gilead); and ombitasvir, paritaprevir, and ritonavir tablets + dasabuvir tablets (Viekira Pak, AbbVie)—hold promise to significantly advance treatment for HCV.

Treatment with Sovaldi has an approximate 90 percent cure rate, in clinical trials, and fewer serious side effects than earlier HCV treatment regimens. The cure rates for Harvoni and Viekira Pak are in the range of 94 percent to 99 percent and are all-oral treatments that are better tolerated and easier to administer.

Accordingly, clinical practice is changing—the new, more effective therapies will likely be used to treat a greater number of people than in the past. However, the cost of treatment will also be high relative to current therapies. The retail price of a single course of Sovaldi combination treatment ranges from \$84,000 to more than \$175,000, depending on the specific treatment regimen. Harvoni is priced at \$94,500 for a typical course of treatment, although in some cases it can be taken for a shorter period and does not require combined treatment with other drugs, such as interferon or ribavirin. And the most recently approved option, Viekira Pak, is priced at about \$83,300. The prospect of treating a larger number of people at a higher cost presents a challenge to state Medicaid and corrections budgets, which could experience

significantly greater near-term expenditures than would have been required for the older therapies. In June 2014, the National Governors Association Center for Best Practices convened a roundtable of experts to discuss opportunities and challenges presented by these treatments and other high-impact options in the pharmaceutical pipeline.

A frequently cited calculation offered by Express Scripts, a private firm that manages pharmaceutical benefits, indicates that state governments could pay as much as \$55 billion over an indefinite period to treat the Medicaid and incarcerated population currently infected with HCV. Underlying that calculation are assumptions about the number of people whose treatment would be paid for by the states—750,000—and the price paid for the treatment—about \$85,000 for each person at the retail price for Sovaldi and about \$66,000 for the same drug for those enrolled in Medicaid. The lower price for people treated under Medicaid reflects the 23 percent discount required by law.

A considerable degree of uncertainty surrounds each assumption underlying the \$55 billion calculation that, when accounted for as a whole, suggests a lower demand on state budgets, particularly in the next several years. For example, the IMS Institute for Healthcare Informatics, a private research group that tracks and forecasts prescription drug sales, estimates that about 450,000 people in the United States will be treated with the new HCV drugs over the five-year period (2014–2018), or about 100,000 each year. Assuming that state programs cover half of those treated and that drug prices do not vary over the period, the five-year cost estimate is about \$18 billion for state programs (an average of \$3.6 billion per year).

The contrast between those estimates reveals two dimensions of uncertainty: the number of people treated for whom states will pay and the number of years over which treatment is spread. Clinical practice suggests a higher number sooner, as does wider testing for the presence of the infection in the general population, and in those for whom states are responsible. Experts differ on treatment recommendations, however. Some suggest a public health intervention approach that would make the drug available to the largest number of infected individuals. Others suggest that the current standard of care, which gives priority treatment to individuals who have advanced liver disease or who are at high risk of developing complications, is appropriate. Still others recommend intermediate positions. Although more restrictive policies would be consistent with past practice, policies that delay treatment of HCV-infected individuals could potentially be challenged in court.

The price of the drugs, however, is an uncertainty not accounted for in either calculation. Competition among the two companies currently providing HCV treatments and at least one more potential entrant to the market appears to be lowering the cost of treatment. Firms currently producing HCV drug treatments already are negotiating discounts for pharmaceutical benefit management firms in exchange for preferred status. Express Scripts is reported to have reached such an agreement for a discount on AbbVie's Viekira Pak in exchange for placing the drug regimen on its preferred list of covered drugs and permitting physicians not specializing in liver disease to prescribe the drug. Similarly, CVS is offering exclusive coverage of the Gilead drug treatments, covering Viekira Pak only when medically necessary. Most recently, Blue Shield of California moved Viekira Pak to preferred status. Those competing agreements appear to have stimulated further discount negotiations between states and the manufacturers, resulting in substantial reductions to treatment costs in some states.

Participants in the expert roundtable offered a range of options and strategies for governors to consider as they develop coverage policies regarding HCV treatments. The panel considered three aspects of the issue:

- **The coverage of newly approved treatments.** State Medicaid programs have the authority to set limits on certain drugs to control costs, ensure correct medical use, and deter fraud and abuse or overuse of certain drugs.
- **The cost of newly approved treatments.** Roundtable participants suggested ideas for increasing states' power to negotiate with manufacturers—for example, through state purchasing cooperatives.
- **Future high-impact drugs.** Experts discussed the current trend toward specialty drug products for common or complex diseases, which are expected to increase pharmaceutical costs significantly, in part because some of them are designed to supplement rather than supplant current therapies.

Introduction

On June 19, 2014, the National Governors Association Center for Best Practices (NGA Center) convened an expert roundtable to discuss the opportunities and challenges presented by new hepatitis C virus (HCV) treatments and other pharmaceutical therapies known as *high-impact drugs*. Such drug treatments share the common characteristics of being more effective than prevailing drug therapies, often resulting in a change in clinical practice that includes treating many more people and being much more expensive. Experts participating in the roundtable included state health care leaders, scientists, national health care experts, subject-matter experts from insurance and pharmaceutical companies, Medicaid and corrections legal experts, and senior staff from the U.S. Department of Health and Human Services (HHS) and the U.S. Department of Veterans Affairs (VA). This

paper summarizes the expert roundtable discussion supplemented by NGA Center research to flesh out the background information and strategies discussed.

An estimated 3.2 million people in the United States are infected with HCV, approximately half of whom are unaware of their status. More than 750,000 receive health care services through Medicaid or state prison systems.¹ By some accounts, both of those estimates are low.² Because the progression of HCV is slow, symptoms can take up to three decades to develop. The more widespread testing of at-risk populations and the improved efficacy of the new drug treatments could increase the number of people in the population diagnosed and treated.

The drug sofosbuvir (Sovaldi, Gilead Sciences, Foster City, Calif.) was the first in a class of direct-acting antiviral agents (DAAs) currently on the market that hold the promise of a cure for chronic HCV. The drug received U.S. Food and Drug Administration (FDA) approval on December 6, 2013. Treatment with Sovaldi, in combination with other needed drugs, has an approximate 90 percent cure rate, in clinical trials, and fewer serious side effects than seen with previous HCV treatments.³

Recently, two competitor treatments have entered the U.S. market. Ledipasvir + sofosbuvir (Harvoni), also manufactured by Gilead, gained FDA approval on October 10, 2014, as the first once-daily oral, interferon-free treatment. An all-oral combination

treatment consisting of ombitasvir, paritaprevir, and ritonavir tablets + dasabuvir tablets and marketed as Viekira Pak (AbbVie, North Chicago) received FDA approval on December 19, 2014. Both treatments show higher cure rates: 94 percent to 99 percent, in clinical trials, for the most common form of HCV. Other drug therapies are anticipated by 2016.

Treatments are expensive: the retail price of a single course of Sovaldi combination treatment ranges from \$84,000 to more than \$175,000, depending on the specific treatment regimen.⁴ The announced prices for Harvoni, at \$94,500 for a typical course of treatment (in some cases, the drugs can be taken for a shorter period at reduced cost), and Viekira Pak, at \$83,319 for a 12-week course, are similarly expensive.⁵ Price and coverage negotiations between AbbVie and the United States' largest pharmacy benefit manager, Express Scripts, have resulted in a reduced price for the manager's covered beneficiaries.⁶ The prices charged by Gilead in the United States for both of its drug treatments are considerably higher than those that will be charged in other countries. Developed countries, such as Germany, Canada, and the United Kingdom, receive a significantly discounted price on Sovaldi, and developing countries see an even steeper discount. The price of a course of treatment in Egypt, for example, is \$900. In addition, Gilead has reached licensing agreements with manufacturers in India to distribute a generic version of the drug to 91 developing countries, likely at no more than 1 percent

¹ Stephen Miller, "State Governments May Spend \$55 Billion on Hepatitis C Medications," Express Scripts, posted July 17, 2014, [http://lab.express-scripts.com/insights/specialty-medications/state-governments-may-spend-\\$55-billion-on-hepatitis-c-medications](http://lab.express-scripts.com/insights/specialty-medications/state-governments-may-spend-$55-billion-on-hepatitis-c-medications) (accessed January 25, 2015).

² Jeffrey A. Tice, Daniel A. Ollendorf, and Steven D. Pearson, *The Comparative Clinical Effectiveness and Value of Simeprevir and Sofosbuvir in the Treatment of Chronic Hepatitis C Infection* (Institute for Clinical and Economic Review, April 2014), http://ctaf.org/sites/default/files/assessments/CTAF_Hep_C_Apr14_final.pdf; and Stephen Miller, "State Governments May Spend \$55 Billion on Hepatitis C Medications."

³ Jake Liang and Marc G. Ghany, "Editorial: Therapy of Hepatitis C—Back to the Future," *New England Journal of Medicine* (May 2014): 1–5, <http://globalhealth.med.ucla.edu/programs/therapyofhepc.pdf>.

⁴ Eric Palmer, "Gilded Says Payers Feel Comfortable with the Cost of Sovaldi," FiercePharma, <http://www.fiercepharma.com/story/gilead-says-payers-feel-comfortable-cost-sovaldi/2014-02-14> (accessed September 10, 2014); and Jeffrey A. Tice, *The Comparative Clinical Effectiveness and Value of Simeprevir and Sofosbuvir*.

⁵ Jonathan D. Rockoff, "Gilead Gets U.S. Approval to Sell New Hepatitis C Drug," *The Wall Street Journal*, October 10, 2014, <http://online.wsj.com/articles/gilead-gets-u-s-approval-to-sell-new-hepatitis-c-drug-1412963083> (accessed October 12, 2014).

⁶ Andrew Pollack, "AbbVie Deal Heralds Changed Landscape for Hepatitis Drugs," *The New York Times*, December 22, 2014, <http://www.nytimes.com/2014/12/22/business/pharmacy-deal-heralds-changed-landscape-for-hepatitis-drugs.html?emc=eta1&r=0> (accessed December 22, 2014); and Matthew Herper, "Drug Price War Takes Pharma Back to the Future," *Forbes*, December 22, 2014, <http://www.forbes.com/sites/matthewherper/2014/12/22/drug-price-war-takes-pharma-back-to-the-future> (accessed December 22, 2014).

of the price charged in the United States.⁷

Experts point out that Sovaldi (and its competitors) has significantly improved treatment for HCV. Nevertheless, those present at the NGA Center roundtable, as well as those within the larger clinical and public health communities, differ in their treatment recommendations (for that drug). Guidance from practitioner associations, FDA, and others is evolving as new data and additional treatments become available.⁸ Some experts suggest taking a public health intervention approach that would include increased screening for infection and widespread access to new treatments to reduce the spread of infection. Others suggest continuing the current standard of care. Still other experts suggest that restricting treatment to individuals who have liver disease or to those at high risk for liver-related or associated complications resulting from HCV is a reasonable course because newer, more effective drug therapies are anticipated and resources are currently not available to treat a larger population.⁹ Intermediate positions emphasize other considerations, such as prescribers adhering strictly to FDA-approved use, whether the patient is ready to receive (and adhere to) treatment, and ensuring that the patient is not actively using drugs or alcohol. Indicative of the evolving approaches, at least one professional association that had recommended liberal use of Sovaldi has updated its guidance to

reflect the interaction between treatment options and significant resource challenges.¹⁰

An upper range of the cost to states for providing treatment with the new HCV drug therapies to the populations for which they are financially responsible is provided by a state-by-state calculation produced by Express Scripts, a firm that manages pharmaceutical benefits. That calculation indicates that states collectively could spend as much as \$55 billion over an indefinite period of time on the most common Sovaldi + ribavirin treatment regimen.¹¹ That estimate assumes treatment of all HCV-infected individuals and a 23 percent “Medicaid best price” discount for state Medicaid programs. A recent analysis by the IMS Institute of Healthcare Informatics, a private research group that tracks and forecasts prescription drug sales, estimated that approximately 15 percent of the HCV-infected population will receive treatment by 2018.¹² Assuming that half of those treated are covered by state programs and that drug prices do not vary over the period of analysis, a relatively conservative five-year cost estimate is about \$18 billion for state programs (an average of \$3.6 billion per year). The expenditures could be higher if more than 3.2 million people have the disease and are treated, or lower if not all of those estimated to be treated actually receive treatment or if the price of treatment falls. Since Sovaldi has become available, states already

⁷ Avik Roy, “The Sovaldi Tax: Gilead Can’t Justify the Price It’s Asking for Hepatitis C Therapy,” *Forbes*, June 17, 2014, <http://www.forbes.com/sites/theapothecary/2014/06/17/the-sovaldi-tax-gilead-cant-justify-the-price-its-asking-americans-to-pay> (accessed December 12, 2014).

⁸ American Association for the Study of Liver Disease, the Infectious Disease Society of America, and the International Antiviral Society—USA, *Recommendations for Testing, Managing, and Treating Hepatitis C*, <http://www.hcvguidelines.org/fullreport> (accessed January 25, 2015); and American Association for the Study of Liver Disease, “AASLD Statement on HCV Guidance,” <http://www.aasld.org/practiceguidelines/Pages/AASLDStatementonHCVGuidance.aspx> (accessed August 16, 2014).

⁹ U.S. Department of Veterans Affairs, *Chronic Hepatitis C Virus (HCV) Infection: Treatment Considerations from the Department of Veterans Affairs National Hepatitis C Resource Center Program and the Office of Public Health*, May 13, 2014, <http://www.hepatitis.va.gov/pdf/2014hcv.pdf>. (accessed January 25, 2015).

¹⁰ American Association for the Study of Liver Disease, *Recommendations for Testing, Managing, and Treating Hepatitis C*.

¹¹ Jason Millman, “The Drug That’s Forcing America’s Most Important—and Uncomfortable—Healthcare Debate,” *The Washington Post Wonkblog*, entry posted July 24, 2014, <http://www.washingtonpost.com/blogs/wonkblog/wp/2014/07/24/the-drug-thats-forcing-americas-most-important-and-uncomfortable-health-care-debate> (accessed September 15, 2014); Stephen Miller, “State Governments May Spend \$55 Billion on Hepatitis C Medications”; and Center on Budget and Policy Priorities, “Policy Basics: Where Do Our State Tax Dollars Go?” <http://www.cbpp.org/cms/?fa=view&id=2783> (accessed December 30, 2014).

¹² IMS Institute for Healthcare Informatics, *Global Outlook for Medicines* (Parsippany, NJ: November 20, 2014): 31, <http://www.imshealth.com/portal/site/imshealth/menuitem.762a961826aad98f53c753c71ad8c22a/?vgnextoid=266e05267aea9410VgnVCM10000076192ca2RCRD&vgnnextchance=a64de5fda6370410VgnVCM10000076192ca2RCRD> (accessed December 29, 2013).

are reporting significant increases in expenditures for HCV treatments.

Although the breakthrough of Sovaldi and its successors has changed the clinical framework for how HCV is treated—from managing a chronic disease to the possibility of eradicating a virus—the cost per person for an initial course of treatment with Sovaldi has been difficult for states to absorb. That cost is projected to continue significantly burdening state budgets into the near future, even when taking into account the diverted cost of treating HCV-related liver complications (a large portion of which is attributable to patients aging into Medicare, and, therefore, the state is not a beneficiary of those savings).¹³ Some experts highlight that the price charged is likely commensurate with the price of drug therapies used to treat HCV before the introduction of Sovaldi—by some calculations, substantially less than the full cost of treating a patient who has experienced liver damage and other complications resulting from HCV infection. An estimated 60 percent to 70 percent of infected individuals will eventually develop chronic liver disease, 5 percent to 20 percent will develop cirrhosis, and 1 percent to 5 percent will die of liver cancer or cirrhosis.¹⁴ As HCV reaches more advanced stages, the cost of care increases considerably.¹⁵ The cost of treating liver cancer in 2010 was estimated to be between \$23,000 and \$44,000; the average cost of a liver transplant in 2010 was estimated to be \$200,000.¹⁶ But the anticipated uptake of the new DAA treatments is substantially higher than in the

past because the new treatments are more effective, easier for patients to tolerate, and easier for health care providers to administer.¹⁷ In particular, individuals who were unresponsive to prior treatment regimens, had experienced debilitating side effects, or had previously delayed treatment are expected to form a substantial base of demand for the new treatments. Thus, it is both the price and the quantity of DAAs expected to be prescribed over the next few years that impose such a significant burden on states.

Consistent with those observations, Gilead reported that prescriptions of Sovaldi between December 2013 and June 2014 exceeded 80,000 patients and were primarily responsible for more than \$6 billion of its antiviral product sales by the end of the second quarter of 2014.¹⁸ Projections show that Gilead stands to achieve nearly \$12 billion in HCV drug sales worldwide in 2014, making it the most lucrative drug launch in history.¹⁹ The availability of Harvoni, Viekira Pak, and a Merck treatment expected to gain FDA approval by 2016 is expected to spark a further increase in demand from those who have HCV but have delayed treatment in anticipation of new therapies that have fewer side effects (a process commonly referred to as *warehousing*).

How competition among those products will affect price has been an open question, and, of course, lower prices would spur the quantity demanded. Some experts do not anticipate that competition will significantly reduce the price of treatments in the United States because pharmaceutical companies have not historically adopted

¹³ Jake Liang, “Editorial: Therapy of Hepatitis C—Back to the Future”; Kathryn Fitch et al., *Health Care Reform and Hepatitis C: A Convergence of Risk and Opportunity* (Milliman, Inc., NY, 2013), <http://us.milliman.com/uploadedFiles/insight/2013/convergence-of-risk-and-opportunity.pdf> (accessed July 18, 2014); and Jeffrey A. Tice, *The Comparative Clinical Effectiveness and Value of Simeprevir and Sofosbuvir*.

¹⁴ Centers for Disease Control and Prevention, “Hepatitis C Information for Health Professionals,” <http://www.cdc.gov/hepatitis/HCV/HCVfaq.htm#section1> (accessed December 29, 2014).

¹⁵ Kathryn Fitch et al., *Health Care Reform and Hepatitis C*.

¹⁶ A.C. El Khoury et al., “Economic Burden of Hepatitis C—Associated Diseases in the United States,” *Journal of Viral Hepatitis* 19 (March 2012): 153–160, <http://www.natap.org/2012/HCV/j.1365-2893.2011.01563.x.pdf> (accessed January 25, 2015).

¹⁷ Although experts generally expect demand to be high, no reliable quantitative estimates of the size and pace of future demand currently exist.

¹⁸ Gilead, “Gilead Sciences Announces Second Quarter 2014 Financial Results,” Press Release, July 23, 2014, <http://www.gilead.com/news/press-releases/2014/7/gilead-sciences-announces-second-quarter-2014-financial-results> (accessed August 6, 2014).

¹⁹ Deena Beasley, “RPT-Gilead to Raise Price for New Hepatitis C Drug Above \$84,000,” *Reuters*, <http://www.reuters.com/article/2014/09/13/gilead-sovaldi-idUSL1N0RD2PC20140913> (accessed October 8, 2014).

strategies based on strong price competition.²⁰ Other experts predict that the competing drug companies will lower their prices in pursuit of a higher market share, although by how much remains unclear.²¹ Most recently, Express Scripts opened the door for price competition by successfully negotiating a reduced price (not disclosed but rumored to be substantial) for Viekira Pak in exchange for covering people regardless of disease stage and allowing nonspecialists to prescribe the drug treatment. In addition, Express Scripts plans to exclude Sovaldi and Harvoni from its national preferred formulary, which covers 25 million people.²² CVS countered with exclusive coverage of the Gilead drugs on several of its formularies under a reportedly similar arrangement.²³ Those developments are influencing state Medicaid negotiations with the manufacturers, resulting in substantial discounts to some states.²⁴

States' Challenges in Developing Policies for High-Impact Drugs

The combination of high price per treatment and the number of people who are seeking or might seek treatment makes it clear that Sovaldi and similar drug treatment therapies will have a large effect on state budgets. Governors have the tough job of evaluating the opportunity costs and associated risks that come with pharmaceutical coverage policies for new drugs for which their state will pay. Governors must weigh the benefits of covering prescription drugs against their responsibility to be good fiscal stewards of programs under their purview while balancing all the other needs of their state residents. When exercising options to limit drug coverage, they must navigate certain constraints

imposed under federal law (see Appendix A on page 15).

A key issue that has emerged is managing the substantial number of HCV-infected individuals who have been awaiting treatment in anticipation of newer therapies. On one hand, delaying treatment for other individuals in the early stages of HCV infection would help manage this volume and is within both historical and current standards of care. On the other hand, delaying treatment might provoke legal challenges because of several factors that emphasize expanded coverage with the new therapies:

- HHS has developed a strategic action plan, including expanded screening recommendations for HCV in adults born between 1945 and 1965, those at peak risk for having contracted HCV, and improved linkage to treatment;
- The Centers for Medicare & Medicaid Services (CMS) will cover HCV screenings for adults at high risk or who were born between 1945 and 1965;
- The U.S. Preventive Services Task Force, an independent group of national experts in prevention and evidence-based medicine, gives a “B” rating to screening for HCV in adults at high risk or born between 1945 and 1965, which suggests that screening is indicated for these populations; and
- Stakeholder groups are calling for a more public health approach for identification and eradication of infectious diseases.

²⁰N.H. Afdhal et al., “The New Paradigm of Hepatitis C Therapy: Integration of Oral Therapies into Best Practices,” *Journal of Viral Hepatitis* 20 (2013): 745–760, <http://www.ncbi.nlm.nih.gov/pmc/articles/PMC3886291/pdf/jvh0020-0745.pdf> (accessed January 25, 2015).

²¹ Tracy Staton, “Don’t Worry About a Hep C Pricing War, Analysts Tell Gilead-Watchers,” FiercePharma, <http://www.fiercepharma.com/story/dont-worry-about-hep-c-pricing-war-analysts-tell-gilead-watchers/2014-08-20> (accessed October 9, 2014); Deena Beasley and Ben Hirschler, “Doctors Welcome Hepatitis C Drug Rivals, Gilead Still Leads,” *Reuters*, <http://www.reuters.com/article/2014/04/11/us-hepatitis-gilead-sciences-idUSBREA3A0CL20140411> (accessed October 9, 2014); and Stephen Williams, “An Overview of the Therapeutic Landscape” (paper presented at States Balancing the Opportunities and Costs of Medical Breakthroughs: The Case Study of New Hepatitis C Treatments, Washington, DC, June 19, 2014).

²²Ibid

²³ Andrew Pollack, “AbbVie Deal Heralds Changed Landscape for Hepatitis Drugs”; Matthew Herper, “Drug Price War Takes Pharma Back to the Future,” *Forbes*, December 22, 2014, <http://www.forbes.com/sites/matthewherper/2014/12/22/drug-price-war-takes-pharma-back-to-the-future> (accessed December 22, 2014); and Avik Roy, “The Sovaldi Tax.”

²⁴Peter Loftus, “States Work to Strike Deals for Hep C Drug Discounts,” *The Wall Street Journal* <http://www.wsj.com/articles/states-work-to-strike-deals-for-hep-c-drug-discounts-1422492687> (accessed January 29, 2015).

States pay for health care provided through Medicaid, the state prison system, and their share of state employee health insurance programs. An estimated 750,000 HCV-infected individuals receive health care through Medicaid or the prison system. The prevalence of HCV is estimated to be about 20 percent to 40 percent among the currently incarcerated population, 2 percent among the uninsured, and nearly 3 percent among the dually eligible Medicare and Medicaid population. About half the infected population is undiagnosed, and that group is concentrated among the uninsured.²⁵ The rate of HCV is highest in the Baby Boom generation, and though the majority of the infected population will age into Medicare over the next 10 years, few are currently eligible for Medicare, thus laying the burden of treatment on state health insurance programs and commercial insurers. Of additional concern for states is the current increase in heroin use among young Americans and the potential for a resurgence of HCV infection within that demographic. (The use of injectable drugs is a significant risk factor for HCV.²⁶)

Views differ on whether states will ultimately realize a significant cost savings from an increased uptake of Sovaldi, Harvoni, and Viekira Pak. Findings from modeling of intervention scenarios, including the new HCV treatments, provide additional economic considerations. A recent report from the Institute for Clinical and Economic Review, a nonprofit organization that evaluates evidence of the value of medical tests, treatments, and delivery innovations, provides an example. The report found that if half of the California population infected with HCV was treated over a one-year period with Sovaldi or a similar drug in accordance with FDA-approved treatment regimens, and the treatments were provided at the

current price of the drugs, expenditures by all payers on drugs would increase by \$22 billion in that one year.²⁷ Further, it found that at the five-year horizon, cost offsets (gains from fewer HCV-related diseases) would represent only 10 percent to 20 percent of upfront treatment costs, increasing to just 75 percent of upfront costs at 20 years. Using the new regimens to treat only those individuals who have advanced liver disease, the estimated initial drug expenditure would be about \$7 billion in the first year. In that scenario, cost savings for reducing liver-related complications at five years would be 17 percent of the added drug costs. Cost savings to the statewide health care system would reach \$1 billion at the 20-year mark.

Potential Strategies for States

Considering the budgetary, clinical, and legal complexity facing states making decisions about coverage for the new HCV drugs and other high-impact pharmaceuticals, it is important to approach coverage policies deliberatively and ensure that decisions have a strong footing in the clinical and legal contexts. Currently, many states are working through that process.

Participants in the expert roundtable offered a range of options and strategies for governors to consider as they develop coverage policies regarding HCV treatments and other high-impact drugs. The panel considered three aspects of this issue:

- The coverage of newly approved treatments in public programs under the governor's stewardship (Medicaid and corrections, in particular);
- The cost of newly approved treatments; and
- Future high-impact drugs.

²⁵ Kathryn Fitch et al., *Health Care Reform and Hepatitis C*.

²⁶ Pradip K. Muhuri, Joseph C. Gfroerer, and M. Christine Davies, "Associations of Nonmedical Pain Reliever Use and Initiation of Heroin Use in the United States" (Rockville, MD: Substance Abuse and Mental Health Services Administration, U.S. Department of Health and Human Services, August 2013), <http://www.samhsa.gov/data/2k13/DataReview/DR006/nonmedical-pain-reliever-use-2013.pdf> (accessed January 25, 2015); and Centers for Disease Control and Prevention, "Viral Hepatitis and Injection Drug Users," *IDU HIV Prevention Fact Sheet* (Atlanta, GA: Centers for Disease Control and Prevention, September 2002), http://www.cdc.gov/idu/hepatitis/viral_hep_drug_use.pdf (accessed July 14, 2014).

²⁷ Jeffrey A. Tice, *The Comparative Clinical Effectiveness and Value of Simeprevir and Sofosbuvir*.

Considering Coverage

Several roundtable participants advised governors to consider their coverage policy options with a solid understanding of authorities available to them under federal Medicaid and corrections law (see Appendix A on page 15) to engage in an informed decision-making process about coverage of HCV treatments and similar drugs. Under federal Medicaid law, state Medicaid programs have leeway to:

- Set limits to certain drugs to control costs;
- Ensure correct medical use; and
- Deter fraud and abuse or overuse of certain drugs.

State approaches to drug purchasing and use policies for Sovaldi are evolving and vary for several reasons. First, current clinical guidelines are inconsistent among professional associations and researchers because of different interpretations of efficacy studies to date. For example, guidelines from the American Association for the Study of Liver Diseases (AASLD) and recommendations from the Oregon Health & Science University–based Medicaid Evidence Based Decisions (MED) Project differ significantly.²⁸ Several state approaches, such as the one used in **Arkansas**, are aligned with recommendations from the MED Project (see the Arkansas box below). Second, some state approaches focus on the short-term cost of the new DAAs, but others are incorporating a longer-term view in anticipation of future treatment options (see the Minnesota box below). Finally, in Medicaid expansion states, the increased continuity between the Medicaid and corrections health systems affects state HCV treatment approaches. Before the Patient Protection and Affordable Care Act (ACA), previously incarcerated individuals were often uninsured upon reintegration into the community. Now, in states that have expanded Medicaid, those individuals generally meet new eligibility criteria for Medicaid. Because more than

10 million Americans cycle in and out of prisons and jails each year, state prison and Medicaid systems are confronted with managing the public health effects of a population that churns between health coverage provided through the prison system and Medicaid.²⁹

Roundtable participants emphasized that after consulting with legal and clinical experts, states could use a deliberative and transparent process—for example, a drug utilization review (DUR) process that involves an evaluation of a drug’s use to determine its medical and cost-effectiveness—to be good stewards of their Medicaid program as required under federal law. Governors could then consider using one or more of the following policy levers available to them:

- Prior authorization to ensure that a drug be used only in accordance with its approved labeling;
- Drug formularies and preferred-drug lists to limit the state’s payments to those drugs that are medically necessary;
- Restriction on coverage of a high-impact drugs only to prescriptions written by a medical specialist (for example, only liver specialists would be permitted to prescribe hepatitis medications); and
- Imposition of copayments for prescription drugs as a way to manage appropriate use (see Appendix A for details).

When reviewing and interpreting the evidence during a DUR process and establishing criteria for prior authorization, state policymakers should rely on diverse sources of information. For example, sources often cited include FDA, the Centers for Disease Control and Prevention (CDC), AASLD, the Infectious Disease Society of America, VA, and the Oregon Health & Sciences University MED Project. Such sources can provide different interpretations

²⁸ American Association for the Study of Liver Disease, *Recommendations for Testing, Managing, and Treating Hepatitis C*; and Allison Leof et al., *Sofosbuvir for the Treatment of Hepatitis C and Evaluation of the 2014 American Association for the Study of Liver Diseases Treatment Guidelines*, (Center for Evidence-based Policy, Oregon Health & Science University, May 2014), http://www.ohsu.edu/xd/research/centers-institutes/evidence-based-policy-center/med/upload/Sofosbuvir_for_HepatitisC_FINALDRAFT_6_12_2014.pdf (accessed July 3, 2014).

²⁹ Michael Ollove, “National Strategy Needed to Eliminate Hepatitis C,” *The National Memo*, <http://www.nationalmemo.com/national-strategy-need-eliminate-hepatitis-c-expert-says> (accessed July 18, 2014).

State Examples on the Approach to Hepatitis C Virus Treatment

Arkansas

The Arkansas Medicaid program is monitoring the evolving therapeutic options for HCV. Based on review of new HCV treatment regimens, Arkansas has concluded that additional data are needed to determine the long-term clinical outcomes of these newer therapeutic options. As a result, the Arkansas Medicaid program has elected to maintain the current HCV standard-of-care treatment regimen, which uses pegylated interferon and ribavirin in combination. Sovaldi and simeprevir (Olysio, Janssen Therapeutics), a component of a combination antiviral treatment regimen, require prior authorization. Approval for those treatments is considered on a case-by-case basis.

Minnesota

Minnesota's Medicaid program elected to cover the new DAA agents when used as part of an FDA-approved HCV treatment regimen. Sovaldi and Olysio both required prior authorization and were only approved when used independently as part of the FDA-approved regimen. Minnesota elected not to cover the Sovaldi/Olysio combination. Through Minnesota's drug review process, fairly flexible criteria were established to grant prior authorization for the use of Sovaldi, which require a patient to be at least 18 years old and to have a diagnosis of chronic HCV. Initially, prescribers requested Sovaldi most frequently for patients who were classified as having a more advanced stage of disease or had a genotype that could be treated with an interferon-free regimen.

Minnesota also has launched an evaluation effort to collect data on patients being prescribed new FDA-approved treatment regimens for HCV to determine which factors might be associated with a higher sustained virulent response (SVR) rate (a measure of virus cleared from the body), such as patient profile and disease stage at the time of treatment, type of provider prescribing, quantity of treatment dispensed, and other factors that might affect a patient's response to the treatment therapy. The data also will position Minnesota to monitor and evaluate its current drug utilization policies and to adjust them as necessary.

With the launch of the newer interferon-free regimens (Harvoni and Viekera Pak), demand is anticipated to increase, especially for patients with earlier forms of disease. At this time, Minnesota is intending to approve requests for FDA-approved regimens used in individuals with more advanced forms of the disease. Minnesota is also planning to implement a requirement for a readiness-to-treat assessment, which will consider social/environmental factors and ensure the patient is likely to be able to complete treatment successfully. Minnesota continues to collect and analyze data and continues to work with the manufacturers to achieve more favorable pricing. Minnesota's ultimate goal is to successfully negotiate a price that is low enough to allow broader access to treatment, particularly for individuals in earlier stages of the disease.

of clinical evidence, on-label use, development of readiness criteria, and protocols to assign priorities among patients.

Governors could consider delegating senior staff—for example, the Medicaid medical or pharmacy director or the corrections medical director—to engage with key stakeholders, such as specialty provider groups, to interpret clinical guidelines and the results of DURs and to understand how those elements translate into anticipating use and uptake.

Governors can partner with FDA to further develop post-approval reports (directors' reports are required to be publicized within 30 days) so that they include information necessary for state review. Governors also might consider the efficacy of an even more rigorous evaluation process that closely links review of the evidence base for effectiveness, safety, and cost comparisons with treatment decisions, including prior authorization policies for high-impact drugs. For example, Washington uses a deliberative process to make coverage and formulary determinations with the goals of improving health outcomes, transparency, elimination of bias, consistency across technologies under review, and regularity of review. They do so via two programs: the Health Technology Assessment Program (HTAP), for evaluating medical technologies, and the Prescription Drug Program (PDP), for evaluating FDA-approved medications. Both programs prioritize evidence-based decision making informed by comprehensive and scientifically rigorous reviews of the effectiveness and safety of new interventions. Washington State is one of several in a consortium of states under contract with the Pacific Northwest Evidence-Based Practice Center at Oregon Health Sciences University (one of 11 such centers in the United States) which completes such comprehensive reviews of the evidence (note: this center and the Medicaid Evidence Base Decision Project, mentioned above, are different centers within OHSU). For medications, the resulting evaluations inform the PDP determination process, which is a public process with broad representation of experts from across the state.

Rather than make coverage determinations, due to limitation under federal Medicaid law (see Appendix A on page 15), those expert participants evaluate classes of drugs and make evidence-informed determinations about whether drugs within a class are therapeutically interchangeable (or equivalent). Assuming such equivalence then allows the state to focus on getting the best price.

Considering Cost and Price

Roundtable participants also generated a range of ideas for how to manage cost by increasing states' power to negotiate drug prices with manufacturers. For example, some stakeholders suggested that states partner with stakeholder groups, including private-sector health insurers, to negotiate lower prices. Innovative approaches to multistakeholder negotiations might prove fruitful but still need to be identified. One cautionary note: some experts have suggested that Medicaid's "best price" sets a floor that limits how much multistakeholder negotiations can reduce prices. Experts emphasized that "best price" is critical to state Medicaid program viability, as the intent of Medicaid best-price legislation is to ensure that taxpayers get the best discount possible. Moving to a system of negotiating public-sector and private-sector pharmaceutical prices together could jeopardize states' ability to achieve costs savings across all drugs and could lead to higher overall state expenditures.

Increase State Purchasing Power

Many states are interested in entering into larger multistate rebate pools for supplemental drugs to negotiate lower net payments to drug manufacturers. Most states already participate in rebate pools, and although they have been effective in negotiating lower prices for generic drugs such as statins, they have been less effective in negotiating lower prices for new specialty drugs such as Sovaldi. Pharmaceutical companies have little incentive to engage in negotiations around specialty drugs when a therapeutic substitute is unavailable because Medicaid programs are required to cover manufacturers' drugs as part of the Medicaid rebate program. However,

participants speculated (and recent evidence supports that speculation) that combined efforts with larger numbers of states might yield more favorable results through increased negotiating power, nationwide gubernatorial messaging, or a federal engagement effort. The statute gives the Secretary of HHS broad leeway to authorize a state to enter directly into agreements with a manufacturer, and CMS has interpreted that authorization to include multistate agreements, thereby giving states some flexibility in how they choose to organize around multistate supplemental drug rebate pools.³⁰

Work Effectively with Managed Care Organizations

Because Medicaid rebates are now available to managed care organizations (MCOs) under the ACA, states and MCOs could work to increase the transparency around the drug rebate process. States might consider setting MCO drug formularies through contracts to ensure that MCOs use the most cost-effective drugs. If MCOs are unwilling to do so, they might consider using carve-outs as a last resort.

Consider Corrections Options

The prevalence of HCV is highest among the incarcerated population. To increase options available to corrections leadership and medical personnel, who also are constrained by limited resources, participants suggested that governors consider the following points:

- States might be able to leverage entities covered under Section 340B of the Public Health Service Act (340B Program) operating in their states, such as federally qualified health centers and public hospitals, to provide Sovaldi to corrections inmates. The 340B Program requires that drug manufacturers provide outpatient drugs to certain covered entities serving vulnerable and

(usually) low-income populations at a reduced price. Experts are unaware of jails or prisons that are currently eligible to directly purchase drugs through the 340B Program, but discussed several instances of prisons and jails generating 340B drug discounts for their inmates vis-à-vis covered entities. Drugs sold through the 340B Program for Medicaid beneficiaries are typically available at a price below the Medicaid best price. Further guidance to clarify the permissible scope of the 340B Programs has been expected from the Administration for the past three years and could clarify the ability of prisons and jails to generate 340B Program discounts for Sovaldi and other drugs purchased for their inmates. That approach also could allow states to identify specific strategies targeted at a population that churns between the prison health system and Medicaid.³¹

- Governors in Medicaid expansion states could consider evaluating the coordination of care and coverage for incarcerated individuals re-entering the community to ensure continuity of care and that the necessary wraparound services are in place for treatment adherence and other medically necessary situations.³²

Long-Term Considerations for Addressing Cost and Price

There was a robust discussion during the roundtable about potential, longer-term strategies that governors could use to address the budgetary costs of high-impact drugs. Some participants suggested that states consider a “mortgage-like” approach to pay for high-impact drugs that amortizes payment for expensive and effective drugs for the entire period over which the benefits occur. That approach could apply to states directly or to private plans contracting with Medicaid and Medicare and, theoretically, allows states to absorb cost over

³⁰Dennis G. Smith, letter to the state Medicaid director, September 9, 2004, <http://downloads.cms.gov/cmsgov/archived-downloads/SMDL/downloads/smd090904.pdf> (accessed January 25, 2015).

³¹Anne Spaulding et al, “Impact of New Therapeutics for Hepatitis C Virus Infection in Incarcerated Populations,” *HCV Treatment in Incarcerated Populations* 21, no. 1 (February/March 2013): 27–35, <http://www.iasusa.org/sites/default/files/tam/21-1-27.pdf> (accessed January 25, 2015).

³²Ibid.

time and over a larger population. Under that approach, manufacturers would receive periodic payments, allowing states to finance the cost of high-impact drugs over a long period, but it could also support current pricing practices (or potentially higher prices). Like other proposals to extend payments into the future for services delivered today, the approach raises issues about a state's ability to commit to such future payments unless funds are appropriated in the present, which undermines the perceived benefit of a mortgage-like approach. Moreover, drug producers would likely seek compensation for the time value of the delayed payments.

Another option under discussion is the use of risk-based payments for expensive pharmaceutical therapies. In such a scenario, payments to the pharmaceutical company would be linked to improved health outcomes, allowing the pharmaceutical industry to receive payments based on patients' outcomes directly associated with a specific drug therapy. Although some stakeholders suggest that linking payments to improved health outcomes would shift risk from states to the pharmaceutical industry, that outcome would be less pronounced with narrowly defined outcome criteria. To reduce the risk that states bear, governors might want to consider whether the price should apply simply to the drug therapy's effect on a patient's immediate medical condition (such as viral infection) or broader health outcomes (such as preventing the long-term need for a liver transplant). As the latter is more consistent with the cost-benefit arguments that drug manufacturers and others make, governors considering such an approach could establish a clearly defined, agreed-upon process to determine the short-, medium-, and long-term health outcomes for which manufacturers are held accountable. The outcomes should be as broadly defined as possible to ensure that pharmaceutical companies are at risk for the full benefit assumed in the cost-benefit analysis. In addition, if the state were assuming immediate risk (for example, by making payments to the drug company

for the pharmaceutical), the pharmaceutical company should set aside a pool of funds to reimburse the state for any losses should the drug prove ineffective at meeting the long-term, agreed-upon objectives.

Negotiating the unit of risk and payment mechanism could prove too difficult, consequently rendering this solution untenable. Initially, drug manufacturers will seek to be compensated for assuming additional risk, which would increase their asking price. In addition, a federal waiver may be required for some risk-bearing arrangements. The unit of risk for which the manufacturer is held accountable and the funding mechanism would be important elements of a discussion with federal entities that consider granting waivers.

The Future of High-Impact Drugs

Pharmaceuticals used to treat HCV dominate current concerns about the cost of new drug therapies, but experts in the roundtable anticipated several other specialty drug products that have the potential to change clinical practice and be much more expensive than current treatments. Such products include new drug therapies for Alzheimer's disease, cancer, cholesterol, inflammatory disease, and multiple sclerosis. Drugs for those conditions could significantly increase the cost of treatment, in some cases because they are designed to supplement rather than supplant current therapies. In those and other cases, the anticipated shift toward specialty drugs and the number of people living with the complex conditions such agents treat will challenge public and private payers alike. A subset of those future high-cost drugs is expected to be among those for personalized (or precision) medicine and gene therapies. For example, the first gene therapy drug, alipogene tiparvovec (Glybera, uniQure NV)—a treatment for the rare genetic condition lipoprotein lipase deficiency—is set to enter the European Union drug market in spring 2015 at \$1.4 million per course of treatment. It is expected to enter the U.S. drug market in 2018.³³

³³ Ludwig Burger and Ben Hirschler, "Exclusive: First Gene Therapy Drug Sets Million-Euro Price Record," *Reuters*, November 26, 2014, <http://www.reuters.com/article/2014/11/26/us-health-genetherapy-price-idUSKCN0JA1TP20141126> (accessed December 10, 2014).

As shown in Figure 1, a useful framework for policy development includes rigorous information gathering, a transparent policy-development process, and policy implementation, with continuous evaluation and adjustment as evidence indicates. Simple, clear adherence to such a framework can guide policymakers through a transparent and deliberative process to make policy determinations that mitigate risk as governors balance providing adequate, clinically appropriate access to transformative treatments and responsibly managing the integrity of public programs.

To prepare for future high-impact drugs, states would be well-advised to know the drug pipeline, perform routine scanning and interpretation of that pipeline, understand prescriber behavior, and routinely consult specialists.

Know the Drug Pipeline

Roundtable participants emphasized the importance of monitoring the pipeline and analyzing it for a full appreciation of high-impact drugs in the immediate future and trends on the horizon. The sources listed in

Appendix B on page 19 are useful for monitoring the status of the new HCV therapies and other high-impact drugs. Although predicting which drug therapies will enter the market as blockbusters is an imperfect science because of limited access to proprietary business information or unanticipated challenges in the drug approval or drug marketing processes, actionable information can be captured by consulting multiple sources.

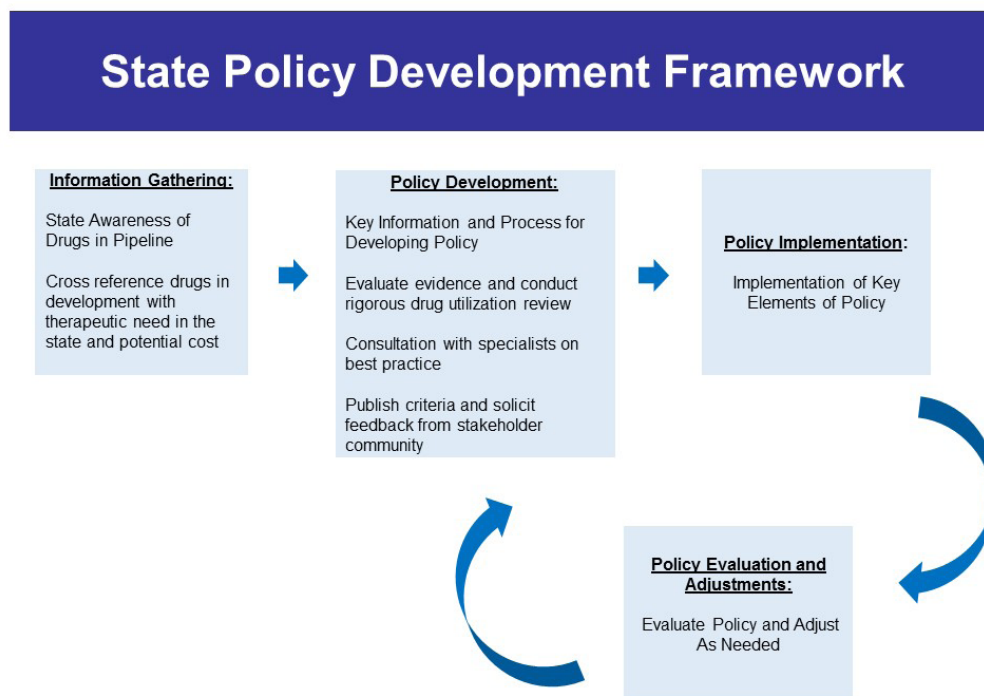
Perform Routine Scanning and Interpretation of the Pipeline

Governors could consider developing a working group or naming a point person, such as the state Medicaid medical director or pharmacy director, to monitor the drug pipeline and anticipate which drugs are coming and when, as well as work with experts to forecast the pricing of those drugs. The point person can engage relevant stakeholders in evaluating the potential utility and effects on treatment and use of drugs under review.

Understand Prescriber Behavior

Governors could consider directing their state agencies or advisors to evaluate patterns in prescribing

Figure 1. State Policy Development Framework



behaviors (for example, provider type, patient type, treatment protocol employed, eligibility criteria, adherence, and outcomes) in the state to identify gaps and inefficiencies within current drug policy and to inform new approaches to delivering quality care, improving health outcomes, and managing cost. Governors could partner with CDC, FDA, and academic medical centers to collect data from state health departments and Medicaid to track prevalence and incidence information, screening, and treatment uptake and response. For example, FDA already has a large-scale effort underway (called *HCV Target*) in

collaboration with the University of Florida and the University of North Carolina to collect observational data on treatment uptake by regimen, SVR, and side effects.

Routinely Consult Specialists

States might consider routine consultation with specialty provider groups to anticipate patterns of prescribing behavior for classes of drugs in the pipeline (for example, innovations in oncology treatments). Such consultation would inform a deliberative, proactive process for approaches to utilization policies.

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Appendix A. Federal Requirements for Outpatient Drug Coverage for Medicaid Beneficiaries and Inmates

States may choose whether to provide outpatient drug coverage for most Medicaid beneficiaries; all states currently provide that coverage.³⁴ By opting to provide prescription drug coverage, however, states are generally required to cover most outpatient drugs marketed by drug manufacturers that have entered into rebate agreements when the drugs are dispensed for medically accepted indications. Although states have some flexibility to manage this coverage, such as imposing utilization controls, these options are subject to the limitations defined under federal law.

Medicaid Outpatient Drug Coverage Requirements

All Medicaid programs receive rebates from drug manufacturers for brand-name and generic products. Generally, for Medicaid to cover an outpatient drug, drug manufacturers must enter into a rebate agreement with the Secretary of HHS (or with a state if so authorized by the secretary).³⁵ Under that agreement, the drug manufacturer must provide rebates to states that in effect result in discount prices for outpatient drugs dispensed to Medicaid beneficiaries.³⁶ In 2014, discounts ranged from 13 percent of the average manufacturer price (AMP) for generic drugs to the higher of 23.1 percent of AMP, average price of drugs sold to retail pharmacies, or best price obtained on the private market for brand-name drugs.³⁷ States may also enter into multistate supplemental drug rebate pools to leverage bargaining and purchasing power with manufacturers and receive additional rebates with approval from CMS. Currently, 45 states participate in this program.³⁸

For an outpatient drug to be covered under Medicaid, drug manufacturers are required to enter into two additional agreements: a pricing agreement with the Secretary of HHS for the program established under Section 340B of the Public Health Service Act and a master agreement with the Secretary of VA to apply the Federal Supply Schedule.³⁹ The 340B Program requires that drug manufacturers provide outpatient drugs to certain covered entities at a reduced price (typically, below that of Medicaid best price). To be eligible to receive 340B Program–purchased drugs, eligible patients must receive health care services other than drugs from the 340B Program–covered entity, with the exception of patients receiving drug therapies in programs such as AIDS drug purchasing assistance programs.⁴⁰ Federal law prohibits duplicate discounts; accordingly, if drugs dispensed to Medicaid beneficiaries qualify for the 340B discount, states are prohibited from seeking a Medicaid rebate for those drugs.⁴¹ As independent entities, jails and prisons generally are not eligible for the 340B Program.

Managing Outpatient Drug Coverage

Under federal law, states have some flexibility in managing Medicaid coverage of certain outpatient drugs to control costs, ensure correct medical use, and deter fraud and abuse or overuse. States have four levers for managing

³⁴ States that have expanded their Medicaid program to cover low-income adults must provide an alternative benefit package (ABP) to this population. Although ABPs must cover outpatient drugs, states are not required to cover all outpatient drugs covered under rebate agreements for the expansion population; instead, they must cover at least one drug in each class.

³⁵ *Payment for Covered Outpatient Drugs*, 42 U.S. Code, 1396r-8.

³⁶ In general, the rebate is the higher of a specified rebate percentage or a rebate that is equivalent to the lowest price the manufacturer has offered for the drug during the rebate period; and *Payment for Covered Outpatient Drugs*.

³⁷ The George Washington University, “The Basics: The Medicaid Drug Rebate Program” (National Health Policy Forum, April 2009), http://www.nhpf.org/library/the-basics/Basics_MedicaidDrugRebate_04-13-09.pdf (accessed July 1, 2014).

³⁸ Medicaid.gov, “State Prescription Drug Resources,” <http://www.medicare.gov/Medicare-CHIP-Program-Information/By-Topics/Benefits/Prescription-Drugs/State-Prescription-Drug-Resources.html> (accessed June 30, 2014).

³⁹ *Payment for Covered Outpatient Drugs*.

⁴⁰ Health Resources and Services Administration, “340B Drug Pricing Program,” <http://www.hrsa.gov/opa/index.html> (accessed June 30, 2014).

⁴¹ *Payment for Covered Outpatient Drugs*.

Medicaid prescription drug coverage: prior authorization, drug formularies and preferred drug lists, the DUR process, and cost sharing. States can require prior authorization for use of a prescription drug before it is dispensed for any medically accepted indication. Drug formularies and preferred drug lists are mechanisms to exclude certain types of drugs or drug classes when permissible under federal law. The DUR process involves prospective and retrospective evaluation of the use of pharmaceuticals measured against predetermined standards to monitor the appropriateness of such use, improve quality, and “conserve program funds or personal expenditures.” States also may impose copayments for prescription drugs as a way to manage appropriate use.

Prior Authorization

Under Medicaid law, states can use prior authorization, which requires approval of a covered outpatient drug before it is dispensed for any medically accepted indication. The system for providing the approval must provide a response to the prior-approval request within 24 hours and provide for the dispensing of at least a 72-hour supply of the covered outpatient drug in an emergency situation.⁴² In addition, states can limit Medicaid coverage for certain drugs through prior authorization based on the medical specialty of the provider prescribing the pharmaceutical (for example, only liver specialists can prescribe hepatitis medications), whether the patient seeking the pharmaceutical is “ready” to receive treatment (developing “readiness to treat” clinical criteria), and whether the intended use of the pharmaceutical complies with its FDA approval (meaning, the intended use is on-label).

Drug Formularies and Preferred Drug Lists

Federal law permits states to exclude or restrict Medicaid coverage of certain classes of drugs or their medical uses.⁴³ Beyond these exclusions, states may use drug formularies or preferred drug lists to steer beneficiaries towards utilizing preferred types or classes of drugs. When establishing these formularies states must comply with certain requirements as described below.

Table 1. Federal Requirements for Establishing Medicaid Drug Formularies⁴⁴

The formulary must be developed by a committee consisting of physicians, pharmacists and other appropriate individuals appointed by the governor of the state or the state’s drug use review board.
The formulary must cover outpatient drugs of any manufacturer that has entered into and complies with the Medicaid rebate agreement unless criteria 3 and 4 below are met (or the drug or its use is on the list of excluded drugs).
Covered outpatient drugs can be excluded from the formulary for the treatment of a specific disease or condition for an identified population if: <ul style="list-style-type: none"> • The drug does not have a significant, clinically meaningful therapeutic advantage over other drugs on the formulary for such treatment; and • A written explanation is made public describing the basis for the exclusion.
The state’s Medicaid plan permits coverage of a drug excluded from the formulary through the prior authorization process.

⁴² Ibid.

⁴³ Examples of exclusions are agents when used for (1) anorexia, weight loss, or weight gain; (2) cosmetic purposes or hair growth; and (3) relief of cough and colds.

⁴⁴ *Payment for Covered Outpatient Drugs*.

Drug Utilization Review Process

State Medicaid agencies are required to use a DUR process to ensure that prescriptions are appropriate, medically necessary, and not likely to produce adverse medical results.⁴⁵ A state's drug review process must include both a prospective and retrospective evaluation of the use of pharmaceuticals, and the data collected during these evaluations must be assessed against predetermined standards to monitor the appropriateness of such use. The DUR process takes place in two phases. During the first phase (*prospective DUR*), the state's Medicaid agency monitors prescription drug claims through its electronic monitoring system to identify problems such as therapeutic duplication, drug–disease contraindications, incorrect dosage or duration of treatment, drug allergy, and clinical misuse or abuse. During the second phase (*retrospective DUR*), the state agency must conduct ongoing and periodic examinations of claims and other data, as necessary, to identify patterns of fraud, abuse, overuse, or medically unnecessary care and use corrective action in necessary circumstances. States must also establish drug-use review boards that guide the implementation of the DUR process, including developing standards for monitoring drug use, reviewing the results of the retrospective DUR, and identifying ongoing interventions for physicians and pharmacists targeted at problems identified during the retrospective DUR.

Cost Sharing

States may impose copayments for prescription drugs. For populations below 150 percent of the federal poverty level, such cost-sharing requirements must be “nominal” (up to \$4 for preferred drugs and \$8 for nonpreferred drugs), and states may not apply cost sharing to certain populations (for example, Medicaid-eligible children or Medicaid patients receiving emergency services). Subject to certain limitations, states can impose higher cost sharing for nonpreferred drugs (up to 20 percent of what the agency pays for the drugs).⁴⁶ In addition, federal law prohibits any provider from denying a Medicaid-covered service because of a beneficiary's inability to pay a copayment.

Other Limits

States can exclude coverage of outpatient drugs if the prescribed use is not for a medically accepted indication. In addition, to discourage waste, fraud, and abuse, states can impose a maximum or minimum quantity per prescription or limit the number of refills a beneficiary can receive with respect to drugs in a particular therapeutic class. States can also implement lock-in programs, which limit Medicaid enrollees to specific pharmacies and providers. Such programs help reduce fraud or abuse by individuals who overuse prescriptions or specific pharmacies.

Outpatient Drug Reimbursement Through Medicaid

Federal law establishes the standard for reimbursement for outpatient drugs. Specifically, reimbursement is the lowest of the estimated acquisition cost plus the dispensing fees; for multiple source drugs with three or more therapeutic or pharmaceutical equivalents, the federal upper limit, or state maximum allowable cost; or the usual and customary charge. States do not calculate estimated acquisition cost uniformly.

Medicaid Managed Care Oversight of Outpatient Drug Coverage

All services available under a state plan must be available and accessible to managed care enrollees. Under Medicaid law, it is permissible for managed care plans to carve out or contract services such as prescription drug benefits from their contracts with states and to provide the services either directly or through separate contractors

⁴⁵ *Payment for Covered Outpatient Drugs.*

⁴⁶ *Applicability; Specification; Multiple Charges*, 42 Code of Federal Regulations, sec. 447.53.

known as *third-party administrators*. Many Medicaid MCOs subcontract management of the prescription drug benefit. Payments under the risk-based contracts are set prospectively, regardless of actual experience, and must be actuarially sound. MCOs control their own drug formulary, but prior authorization is subject to state contractual requirements. In addition, the ACA expanded the Medicaid Drug Rebate Program to drugs provided to enrollees in Medicaid managed care plans.

Pharmaceutical Benefits for Incarcerated Populations

The U.S. Supreme Court has determined that states and local governments are required to provide adequate health care services to detained and incarcerated individuals in accordance with the Eighth and 14th Amendments to the U.S. Constitution.⁴⁷ The obligation to provide health care services to state inmates during their incarceration is based on the Eighth Amendment's prohibition against the infliction of "cruel and unusual punishment." Detainees are protected under the 14th Amendment because a failure to provide care would equate to an impermissible punishment of individuals who have not yet been convicted of a crime.

A series of court decisions has established the legal standards by which prisoners can challenge a failure or delay in providing health care services as a violation of the Eighth Amendment. Prisoners must show that the failure or delay to provide care was in "deliberate indifference to serious medical needs" that resulted in "the wanton infliction of unnecessary pain." The deliberate indifference standard requires that the prisoner prove that he or she had a serious medical need, that the defendant knew of that need, and that the defendant (such as the state prison) deliberately failed to provide required treatment for that need or provided it in a reckless manner. The plaintiff must also show that the course of events caused needless suffering or harm or an "objectively serious worsening" of the medical condition. The legal standard is both objective, requiring a finding of the prisoner's serious medical need, and subjective, requiring the defendant to have known of the need or condition and to have understood that his or her actions or failure to act created a considerable risk of causing the prisoner harm, either immediately or in the future.⁴⁸

⁴⁷ *Payment for Covered Outpatient Drugs*.

⁴⁸ *Applicability; Specification; Multiple Charges*, 42 Code of Federal Regulations, sec. 447.53.

Appendix B. Sources of Information About High-Impact Drugs in the Drug Pipeline

Table 2. Resources for Monitoring the Drug Pipeline and Research and Development

Sources	Website
Datamonitor Healthcare monitors the pharmaceutical pipeline, analyzing therapeutic focuses, launch and expiry balances, diversification, and profitability.	http://www.datamonitorhealthcare.com
Express Scripts: The Lab. Reports on drugs in the pipeline and on overall drug trends and evaluates cost and anticipated effect on payers.	http://lab.express-scripts.com
FierceBiotech* provides the latest on biotechnology and the pharmaceutical industry, clinical trials, FDA decisions, and important regulatory issues. It provides a free newsletter that includes this information.	http://www.fiercebiotech.com
Pharma Information & Reports aims to help companies optimize drug discovery and product pipelines through identification of breakthrough research and in-depth evaluation of therapeutic areas.	http://www.phrma.org/profiles-reports
EvaluatePharma provides key industry analysis of the pharmaceutical and biotechnology health care sectors.	http://www.evaluategroup.com/public/EvaluatePharma-Overview.aspx
Wall Street industry analysts, private equity investors, or venture capital organizations operating in the health care space. These sources are particularly adept at and informative about potential prices of high-impact drugs.	n/a

Table 3. Resources for Monitoring Research and Development

Sources	Website
IMS Health is a private information, services, and technology company that applies analytics and proprietary application suites to evaluate health care data on diseases, treatments, costs, and outcomes. The company develops forecasting solutions for life sciences manufacturers ranging from five to 10 years out, by country, and develops interactive forecasting tools that help organizations evaluate a product's likelihood of success, understand macro trends shaping the market, and determine whether a market warrants investment.	http://www.imshealth.com/portal/site/imshealth/menuitem.051a1939316f851e170417041ad8c22a/?vgnextoid=7311e590cb4dc310VgnVCM100000a48d2ca2RCRD&vgnnextfmt=default
ClinicalTrials.gov** is a registry and results database of publicly and privately supported clinical studies of human participants conducted both in the United States and internationally.	http://clinicaltrials.gov
BioCentury Publications provides value-added information, analysis, and data for biotechnology and pharmaceutical companies, investors, academia, and government on the development and sustainability of life science ventures.	http://www.biocentury.com/Home

* Available free of charge.

** Available free of charge.