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March 4, 2016

The Honorable Ron Wyden
Ranking Member
Committee on Finance
United States Senate

The Honorable Charles Grassley
Senator
Committee on Finance
United States Senate

Dear Ranking Member Wyden and Senator Grassley,

The Pharmaceutical Research and Manufacturers of America (PhRMA) appreciates the opportunity to submit comments in response to your request regarding innovative, breakthrough drugs. PhRMA represents the country's leading innovative biopharmaceutical companies, which are devoted to discovering and developing medicines that enable patients to live longer, healthier, and more productive lives.

Breakthrough medicines represent significant scientific advancements that transform the treatment and thus the downstream healthcare costs of complex and costly diseases, such as hepatitis C (HCV), cancer, and cardiovascular disease. Today, more than 7,000 medicines are in development worldwide, of which 70% have the potential to be first in class and 42% are personalized medicines. The biopharmaceutical industry, the most research intensive sector in the United States, has invested over $500 billion in research and development since 2000, striving to conquer disease, provide therapeutic options, and prolong life, bringing hope to millions of patients with serious, unmet medical needs. Continued advances in biopharmaceutical innovation represent the best opportunity to improve health outcomes and control future health care costs. Unlike most health care services, medicines allow patients to avoid costlier services, such as emergency room visits and long-term care.

The cost of innovation is an important discussion. Central to that discussion is the recognition of how the competitive market works to balance affordability and future investments in innovative therapies. While new medicines generate a large share of the medical advances that patients need and our society demands, older medicines remain highly useful, go off patent, and generate even more substantial cost savings into the future. This is one of the reasons that new medicines reach patients year after year, improving health outcomes, while the total cost of medicines remains a stable and small share of health spending. Looking at the cost of new medicines in isolation obscures the savings they produce through better, more effective care and can unintentionally lead to distorted policy conclusions.

PhRMA's submission explores in more detail the value of medicines to patients and the health care system; how the competitive market balances innovation, access, and affordability, so that

the vast majority of new medicines have a modest impact on drug and total health spending; and the need to support the market's rapid evolution toward a value-driven health care system.

The Value of Medicines: Transforming Patient Care & the Health Care System

Tremendous advances demonstrate the important role that medicines play in solving the complex problem of disease. Advances in cancer, human immunodeficiency virus (HIV), and cardiovascular disease provide just a few examples of how medicines are transforming treatment for patients and society:

- Now more than ever, our country is focused on beating cancer. Over the last several decades we have made real progress against this disease, and due to continued scientific progress we are on the cusp of much more significant advances. Since peaking in the 1990s, cancer death rates have declined 23%.[1] Approximately 83% of survival gains in cancer are attributable to new treatments, including drugs.[2] Recent advances in medicines, such as personalized therapies and immunotherapy, are widely recognized as transformative.[3] As of 2014, cancer medicines accounted for roughly 1% of national health spending[4], while spending on other types of cancer care account for several times this amount.[5] Many new, targeted cancer treatments are not only more effective but have lower toxicity and fewer side effects than earlier treatments,[6] which can allow patients to be treated in lower-cost settings of care and continue live productive lives. Many leaders in oncology predict that in the coming decade we will achieve with cancer the same progress as with HIV and acquired immunodeficiency syndrome (AIDS).[7]

- Unlike the early years of the AIDS crisis, HIV is no longer considered acutely fatal, but rather a chronic manageable disease for those receiving appropriate treatment. This dramatic change followed the introduction of highly active antiretroviral therapy (HAART) in the mid-1990s, which have transformed treatment, leading to an 85% decline in death rates and preventing an estimated 862,000 premature deaths in the US.[8] Progress has continued. Just over the past decade average life expectancy increased over 10 years.[9]

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[1] American Cancer Society. Cancer Facts & Figures 2016. Atlanta: American Cancer Society; 2016.
[2] E. Sun, et al., "The Determinants of Recent Gains in Cancer Survival: An Analysis of the Surveillance, Epidemiology, and End Results (SEER) Database," Journal of Clinical Oncology, May 2008 Suppl (Abstract 6616).
[3] Columbus G. "American Society of Clinical Oncology Names Immunotherapy 'Cancer Advance of the Year.'" *Cure.* February 5, 2016.
[4] PhRMA calculations from National Health Expenditure Data.
[5] Newcomer LN. "Innovative Payment Models and Measurement for Cancer Therapy." *Journal of Oncology Practice.* May 2014. Vol 10, No 3. 187-189.
[6] For example: Foluso O, et al. "Ibrutinib as a Bruton Kinase Inhibitor in the Management of Chronic Lymphocytic Leukemia: A New Agent With Great Promise." *Clinical Lymphoma, Myeloma & Leukemia.* Nov 22, 2015.
[7] Abernathy A, et al. "Turning the Tide Against Cancer Through Sustained Medical Innovation: The Pathway to Progress." *Clinical Cancer Research.* February 12, 2014.
[8] Kaiser Family Foundation. The HIV/AIDS Epidemic in the United States. Available at: http://kff.org/hivaids/fact-sheet/the-hivaids-epidemic-in-the-united-states/
[9] Samji H, Cescon A, Hogg RS, Modur SP, Althoff KN, Buchacz K, et al. (2013) Closing the Gap: Increases in Life Expectancy among Treated HIV-Positive Individuals in the United States and Canada. PLoS ONE 8(12): e81355. doi:10.1371/journal.pone.0081355

- Due in part to the benefits of biomedical innovation, between 2001 and 2011 alone the death rate for cardiovascular disease has fallen by 39%.[10] Leading researchers have estimated that just one class of cardiovascular medicines – statins – yielded about 86,000 fewer deaths and 833,000 fewer hospitalizations for heart attack and stroke annually.[11] Most statins are now used as generics, with the advances developed by biopharmaceutical innovators continuing to generate enormous benefits to society at very low cost. At the same time, more can be done. Many patients at risk for cardiovascular disease still face unmet medical needs and more Americans die of cardiovascular disease every year than from all forms of cancer combined. Access to new treatment options would significantly benefit many patients, such as, those who do not respond to or cannot tolerate statins due to side effects.

- Looking ahead, the number of Alzheimer's cases is projected to increase rapidly as Baby Boomers age, resulting in an enormous human and economic cost. If we can achieve treatment advances that delay Alzheimer's by just five years beginning a decade from now, 2.5 million fewer Americans will be afflicted by the disease and we would avoid $367 billion annually by 2050 in costs for long-term care and similar services for persons with Alzheimer's.[12] Alzheimer's remains a major focus of biopharmaceutical research companies despite high risks; since 1998 there have been 123 unsuccessful attempts to develop a medicine for Alzheimer's, and just four approved medicines.[13]

New medicines are not only saving and improving the lives of millions of patients, but also help control overall health care costs by preventing costly complications and hospitalizations, and replacing other medical interventions. A 2013 study by IMS Institute for Healthcare Informatics estimated that the U.S. health care system could save $213 billion annually if medicines were used properly.[14] An article in *Health Affairs* found that just an extra $1 spent on medicines for adherent patients with congestive heart failure, high blood pressure, diabetes and high cholesterol can generate $3 to $10 in savings on emergency room visits and inpatient hospitalizations.[15]

Based on the growing body of evidence about medicines' benefits, the Congressional Budget Office (CBO) recognizes reductions in other medical expenditures associated with increased use of prescription medicines in Medicare Part D. Research indicates that the savings may be three to six times greater than estimated by the CBO for seniors with common chronic conditions like diabetes and hypertension, and less prevalent conditions such as Parkinson's disease.

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[10] American Heart Association and American Stroke Association. Heart Disease and Stroke Statistics – At-a-Glance. Available at: https://www.heart.org/idc/groups/ahamah-public/@wcm/@sop/@smd/documents/downloadable/ucm_470704.pdf

[11] Cutler DM, Long G, Berndt ER, et al. The Value of Antihypertensive Drugs: A Perspective on Medical Innovation. *Health Affairs*. 2007;26(1):97-110.

[12] Alzheimer's Association. "Changing the Trajectory of Alzheimer's Disease: How a Treatment by 2025 Saves Lives and Dollars." https://www.alz.org/documents_custom/trajectory.pdf

[13] PhRMA. *Researching Alzheimer's Medicines: Setbacks and Stepping Stones.* Summer 2015. Available at: http://phrma.org/sites/default/files/pdf/alzheimers-setbacks-and-stepping-stones.pdf

[14] IMS Institute for Healthcare Informatics, "Avoidable Costs in U.S. Healthcare: The $200 Billion Opportunity from Using Medicines More Responsibly," June 2013, http://www.imshealth.com/en/thought-leadership/ims-institute/reports/avoidable-costs

[15] Roebuck MC, et al. "Medication adherence leads to lower drug spending." *Health Affairs. 2011;30(1):99.*

Role of the U.S. Competitive Market & Prescription Drug Lifecycle in Balancing Innovation, Patient Access, and Cost Containment

The dynamics of the private, market-based system in the U.S. promote incentives for continued innovation and patient access to needed medicines while leveraging competition to achieve cost containment. The competitive market is the engine that drives the innovative biopharmaceutical research and development ecosystem. Since 2000, biopharmaceutical companies have brought more than 500 new medicines to the U.S. market, resulting in significant progress against some of the most costly and challenging diseases.[16,17] Yet, spending on retail prescription medicines accounts for the same percentage of health care spending today as in 1960—just 10%.[18] And, total (retail and non-retail) spending on medicines is projected to remain stable as a percentage of total health care spending (13-14%) in the years ahead, even as numerous new medicines are brought to patients.[19] At the state level, Medicaid programs spent just 4.5% of their budgets on prescription drugs, including new medicines, in 2015.[20]

The U.S. biopharmaceutical marketplace promotes innovation and affordability through cost containment that is built into the prescription drug lifecycle. While the price of a medicine may increase or decrease over its lifetime, prices fall dramatically as competition occurs among brand-name medicines, and typically fall even further (up to 80%) with the introduction of generics.[21] For instance, the price of one common statin (atorvastatin, known in the branded form as Lipitor) used to lower cholesterol and prevent cardiovascular disease, dropped by about 92% from 2005 to 2013 when generic alternatives came to market.[22] Meanwhile, the average charge for percutaneous transluminal coronary angioplasty (PTCA) – a procedure to treat cardiovascular disease – increased by almost 66% during that same time period.[23]

The competitive U.S. market is structured to take maximum advantage of savings from brand competition and from generics. Four large, sophisticated pharmacy benefit managers (PBMs) manage over 80% of all prescriptions filled.[24] They use brand competition to obtain discounts from manufacturers and take full advantage of the presence of generics to drive savings. This will continue in the years ahead, as $115 billion of U.S. brand sales are projected to face generic

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[16] US Food and Drug Administration. Summary of NDA approvals & receipts, 1938 to the present. http://www.fda.gov/aboutfda/whatwedo/history/productregulation/summaryofndaapprovalsreceipts1938tothepresent/default.htm. Published January 18, 2013. Accessed February 2015.

[17] US Food and Drug Administration. New drugs at FDA: CDER's new molecular entities and new therapeutic biological products. 2012 – 2015. Available:

[18] Martin AB, et al. National Health Spending in 2012: Rate of Health Spending Growth Remained Low for the Fourth Consecutive Year. Health Affairs, 33 no. 1 (2014): 67-77

[19] Altarum Institute. Center for Sustainable Health Spending Data Brief: A Ten Year Projection of the Prescription Drug Share of National Health Expenditures Including Non-Retail, August 2015, http://altarum.org/sites/default/files/uploaded-publication-files/Non-Retail%20Rx%20Forecast%20Data%20Brief_with%20Addendum.pdf

[20] The Menges Group. 10 Year Medicaid Spending Forecast by State, October 2015.

[21] IMS Institute for Healthcare Informatics. Price Declines after Branded Medicines Lose Exclusivity in the U.S. January 2016. https://www.imshealth.com/files/web/IMSH%20Institute/Healthcare%20Briefs/Price_Declines_after_Branded_Medicines_Lose_Exclusivity.pdf

[22] Atorvastatin, known in the branded form as Lipitor 10mg: IMS National Sales Perspective (NSP) Invoice Price in 2005 (Branded Lipitor) and in 2013 (Generic Atorvastatin).

[23] Data adapted from: HCUP Hospital Charge Database 2005 to 2013, Average Hospital Charges.

[24] Drug Channel Institute Study, January 2015. IMS Institute for Health Informatics Health Study: 2014.

competition between now and 2018.[25] Medicines face some of the most stringent cost controls in health care, evident in the concentration of purchasing power, extensive use of aggressive tactics such as tiered formularies and step therapy. This drives the rapid shift of market share to generics (and, looking forward, to biosimilars); a system with few analogues in other health care sectors.

These cost containment tools go to work quickly when a new medicine is introduced, with the result that in the vast majority of cases, introduction of a new medicine leads to only a modest increase in prescription drug spending and an imperceptible increase in overall health spending. In fact, new brand medicines – products launched in the previous five years – accounted for only 0.21% and 0.25% of total health spending in each year between 2010 and 2013, rising to 0.35% in the outlier year of 2014.[26] Another way to understand the role of new medicines in health costs is to look at the revenue typically earned by newly launched drugs. Data from IMS Health show that 89% of all new drugs launched from 2010-2013 generated less than $500 million of revenue by the end of their second year on the market, and 97% generated less than $1 billion.[27]

In fact, these figures likely *overestimate* the budget impact for new medicines because they are based on data not adjusted for discounts and rebates, and ignore the reality that new medicines often replace spending on older brand and generic medicines and other health care services.[28] For instance, as discussed below, HCV was responsible for large and growing health costs in recent years, though this drew little attention prior to the introduction of a cure. Notably, total spending on all medicines is projected to be roughly the same share of overall health expenditures in 2023 as it was in 2013, before the introduction of new hepatitis C treatments.

HCV provides a recent illustration of the competitive market forces at work. HCV is a serious public health problem with growing disease-related burden and significant associated medical costs. For example, looking at data prior to the introduction of the first of a new generation of treatments—direct acting antivirals—in 2011, one study found that the costs for treating commercially insured patients with HCV averaged 5.5 times the cost of commercially insured patients overall,[29] while another estimated that in absence of a cure, the incremental cost of HCV to the U.S. health care system would be $115 billion over the next decade.[30] At that time, the only available treatment was an injected immunotherapy which cured only half of patients and caused debilitating side effects over the 24 – 48 week course of treatment. Today, as a result of tremendous advances in treatment, new oral treatment options are offering patients cure rates greater than 90% in as little as eight weeks with minimal side effects, for patients with all known

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[25] IMS MIDAS, Market Prognosis, Sep 2014.

[26] PhRMA analysis based on IMS Institute March 2016, and CMS National Health Expenditures Report, December 2015.

[27] PhRMA analysis based on IMS Institute March 2016

[28] B Leinwand, et al. A Case for Market-Based Costs in Determining Cost Effectiveness: The Hepatitis C Example. Published November 2015. Available: http://avalere.com/expertise/life-sciences/insights/new-analysis-finds-cost-effectiveness-models-for-hepatitis-c-drugs-often-ov

[29] Fitch, Iwasaki, Pyenson, Engel, "Health Care Reform and Hepatitis C: A Convergence of Risk and Opportunity," Milliman. December 2013. http://us.milliman.com/uploadedFiles/insight/2013/convergence-of-risk-and-opportunity.pdf

[30] Pyenson B, Bochner A, Cannon R. An Actuarial Approach to the Incremental Cost of Hepatitis C in the Absence of Curative Treatments. Milliman, Inc. September 2015. http://www.milliman.com/uploadedFiles/insight/2015/20150915_Incremental-Cost-of-HCV-White-Paper.pdf

forms of the disease.[31] Researchers project that with current screening guidelines and the availability of today's more effective treatments, HCV will be a rare disease by 2036.[32]

When the first of the new generation of HCV medicines reached patients after being approved by the Food and Drug Administration, PBMs claimed that these life-changing treatments and cures would bankrupt the health care system and wreak financial havoc. According to the CEO of Express Scripts, "What they have done with this particular drug will break the country… It will make pharmacy benefits no longer sustainable. Companies just aren't going to be able to handle paying for this drug."[33] Yet, the public record demonstrates that competition drove deep discounts – average rebates on some products increased from about 22% in 2014[34] to publicly reported discounts now ranging from 40-65%. Competition has also led to lower launch prices. PBM industry leader Express Scripts now states that their aggressive negotiations have saved Americans $4 billion, cured more patients with HCV than any time in history, and that the discounted price makes it affordable to treat all patients with the infection.[35] They have also touted their forceful bargaining as the reason why HCV treatments are less expensive here than in many western European countries.[36]

Competition holding down cost is not unique to HCV medicines. For instance, prior to the launch of proprotein convertase subtilisin kexin 9 (PCSK9) inhibitors, a new type of cholesterol lowering medicine that represents a significant advance in treatment of heart disease, PBMs made alarming claims about their cost – in one account, projecting up to $150 billion-$200 billion per year in spending for these medicines.[37] The Centers for Medicare & Medicaid Services (CMS) Office of the Actuary, however, projected a much more modest impact, based on expected competition leading to discounts and continued widespread use of generic statins.[38] The Actuary's failure to accept alarmist claims about the cost of these new medicines was quickly proven correct, as PBMs made deals to cover the drugs and emphasized that their cost

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[31] Cure rates were pulled from a variety of sources, including FDA labels, company press releases and materials. Reported cure rates vary by treatment population. For a full list of source material for cure rates of HCV medicines see: PhRMA. A decade of innovation in chronic diseases: 2006 – 2016. Available at: http://phrma.org/sites/default/files/pdf/decade-of-innovation-chronic-disease.pdf Published February 2016.

[32] Kabiri M, Jazwinski AB, Roberts MS, Schaefer AJ, Chhatwal J. The Changing Burden of Hepatitis C Virus Infection in the United States: Model Based Predictions. *Annals of Internal Medicine*. 2014;161(3): 170.

[33] Cortez MF. "Express Scripts Raises Pressure on Gilead for Drug Price." *Bloomberg Business*. April 8, 2014.

[34] Silverman, "The Hepatitis C Scorecard: Gilead is Trouncing AbbVie, but at a Price," *Wall Street Journal*, Feb. 12, 2015, http://blogs.wsj.com/pharmalot/2015/02/12/the-hepatitis-c-scorecard-gilead-is-trouncing-abbvie-but-at-a-price/

[35] Express Scripts. The $4 Billion Return on a Promise Kept. January 27, 2015. http://lab.express-scripts.com/lab/insights/specialty-medications/the-4-billion-return-on-a-promise-kept

[36] LaMattina J. For Hepatitis C Drugs, U.S, Prices Are Cheaper Than in Europe. *Forbes*. December 4, 2015. http://www.forbes.com/sites/johnlamattina/2015/12/04/for-hepatitis-c-drugs-u-s-prices-are-cheaper-than-in-europe/#7ced43f564bb

[37] Shrank W, Lotvin A, Singh S, Brennan T. In the Debate About Cost and Efficacy, PCSK9 Inhibitors May Be The Biggest Challenge Yet. Health Affairs Blog. February 17, 2015. http://healthaffairs.org/blog/2015/02/17/in-the-debate-about-cost-and-efficacy-pcsk9-inhibitors-may-be-the-biggest-challenge-yet/

[38] Kelly C. U.S. Drug Spending Will Increase 7.6% in 2015, Including PCSK9 Costs – CMS. *The Pink Sheet*, July 2015.

was "far lower than industry forecasts."[39]

As illustrated by the experience with HCV medicines and PCSK9 inhibitors, multiple companies are often simultaneously competing to research, develop, and secure Food and Drug Administration (FDA) approval of first-in-class treatments. In fact, drug development has become more competitive than ever, both during the R&D process and after medicines are approved, as the race to be the first drug to launch in a new therapeutic class has intensified. 88% of first-in-class medicines launching in 2005-2011 already had a competitor in Phase II testing at time of their launch, compared to 63% for first-in-class drugs launched in 1998-2004. Once launched, the time a drug is alone in its class has continued to shrink, from 4.7 years for drugs approved between 1998 and 2004, to 2.3 years for drugs approved between 2005 and 2011.[40] The rigor of the market's competitiveness and other characteristics is evident in the fact that only two in ten of all approved medicines will produce revenue that exceeds average R&D costs.[41]

Similarly, a recent analysis by a Massachusetts Institute of Technology economist and the IMS Institute finds that increasing market competition has eroded much of the economic profitability of newly launched brand medicines, such that on average financial returns for medicines launched between 2005 and 2009 were insufficient to recoup average R&D and operating costs.[42] Even drugs that succeed at launch may quickly be supplanted as other new brand competitors enter the market, as occurred with first generation HCV medicines. For example, despite initial success, two protease inhibitors launched in 2011 – seen at the time as substantial advances in treatment for HCV – found that they were supplanted by more effective treatments following the introduction of the next generation of medicines in 2013. Thus, despite substantial investment and many years of research and development, competition from newer brands led these medicines to be withdrawn from the market within two years.[43] This underscores the extraordinary risk involved in bringing new treatments to market.

The rapid pace by which new medicines emerged to effectively treat and cure HCV exemplifies how competitive market forces drive innovation in the US health system while also containing costs. It is a dynamic that is described in detail in the report. The substantial unmet need presented by HCV led many companies to undertake the considerable time and investment to bring new medicines to patients. Companies raced amongst one another not only to provide effective treatment options but also to meet remaining unmet patient need. This intense competition led to (1) the development and approval of effective treatments for patients with all forms of the disease—including the most common form affecting 70% of HCV patients (genotype 1) as well as a broad range of rarer forms, (2) significant price reductions that payers

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[39] Express Scripts. "Express Scripts Includes Innovative Cholesterol-Lowering Drugs on National Preferred Formulary." October 6, 2015. http://www.prnewswire.com/news-releases/express-scripts-includes-innovative-cholesterol-lowering-drugs-on-national-preferred-formulary-300155222.html

[40] Tufts Center for the Study of Drug Development. First-in-class drugs in competitive development races with later entrants. *Impact Report.* 2015;17(6).

[41] Vernon JA, Golec JH, DiMasi JA. Drug development costs when financial risk is measured using the fama-french three-factor model. *Health Economics.* 2010;19(8):1002-1005.

[42] Berndt ER, Nass D, Kleinrock M, Aitken M. Decline in Economic Returns from New Drugs Raises Questions About Sustaining Innovations. *Health Affairs.* 2015;34(2):245-252.

[43] Loftus P. Merck will no longer sell its victrelis hepatitis C drug in the U.S. *Wall Street Journal*, Jan 21, 2015, http://blogs.wsj.com/pharmalot/2015/01/21/merck-will-no-longer-sell-its-victrelis-hepatitis-c-drug-in-the-u-s/

acknowledge now make it affordable to provide broad access to patients, and (3) competition that continues to increase as more competitors enter the market.

Today, companies continue to compete to bring more effective and convenient treatment options to patients—including more challenging treatment populations such as those co-infected with HCV and HIV. These gains are occurring in an environment that encourages competing research-based biopharmaceutical companies to take the risk of seeking to be the first to market, while recognizing they might ultimately be second or third, or fail altogether. Focusing only on the first to market medicine risks missing this larger, beneficial competitive dynamic and the incentives needed to sustain it.

Competitive Market Drives the Availability of Extensive Information for Use by Payers

To support informed decision-making, payers rely on a broad range of information, both pre- and post-approval. This information includes the extensive pre-clinical and clinical trial data required to meet FDA's stringent premarket approval standards, systematic reviews conducted by the academic community, "horizon scanning" programs that identify and evaluate data on emerging advances,[44] and, increasingly, data generated internally and externally via electronic datasets.

Payers also review a wide range of evidence on clinical and economic outcomes in making decisions about drug coverage and formulary placement decisions for new treatments, or new indications for existing treatments. For example, the Academy of Managed Care Pharmacy developed a format to guide pharmaceutical manufacturer formulary submissions, including evidence supporting the clinical and economic value of submitted medicines.[45] Payers tout their use of information about a medication's indication, comparison to other treatments, the burden of disease, and relevant treatment guidelines to defend their assessments of medicines and decisions about coverage, tier placement, utilization management, etc. [46] However, some payer decisions appear to prioritize short term costs over longer term benefits and patient interests.

In contrast, most other types of health care treatments are not required to meet a similar standard before being used in clinical practice. Notably, there are also no such standards for payers' changes to delivery, payment and utilization management, which can significantly affect the care patients receive; we may know far less about the impact of these interventions than we do about medicines.[47]

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[44] "Horizon scanning" programs are intended to provide payers, providers and other decision-makers with information that will help them plan for introduction of important new tests and treatments. One such program, the Healthcare Horizon Scanning System, is supported by the Agency for Health Research and Quality (AHRQ). Other programs are operated by payers, commercial sector companies specializing in such information, and private health technology assessment organizations.

[45] Academy for Managed Care Pharmacy. *The AMCP Format for Formulary Submissions. A Format for Submission of Clinical and Economic Evidence of Pharmaceuticals in Support of Formulary Consideration.* Version 3.1. Dec 2012.

[46] See: Express Scripts. *White Paper: Formulary Development at Express Scripts.* Available at: https://www.express-scripts.com/aboutus/formularyinformation/development/formularyDevelopment.pdf; and WellPoint. *Health Technology Assessment Guidelines: Drug Submission Guidelines for Re-evaluation of Products, Indications, and Formulations.* Updated September 2008.

[47] See, for example: Kip Sullivan, "Why We Have so Little Useful Research on ACOs." *The Health Care Blog.* February 16, 2016. http://thehealthcareblog.com/blog/2016/02/16/why-we-have-so-little-useful-research-on-acos/

The wave of HCV treatments approved in 2013 and early 2014 illustrate the amount of information available about medicines in the research pipeline. As early as 2012, researchers were conducting systematic reviews of evidence from clinical trials of the HCV treatments, including the medicine that would later become Sovaldi, and drawing the conclusion that the forthcoming treatments would likely represent a substantial clinical improvement over the standard of care at that time.[48] In June 2013, Agency for Healthcare Research and Quality's Healthcare Horizon Scanning System listed "*Sofosbuvir (GS-7977) for treatment of chronic hepatitis C infection*" as an area of high impact potential,[49] and accurately forecast the list price to be between $75,000 and $85,000. Similarly, a report by Optum, part of UnitedHealth Group, predicted a significant increase in reimbursement claims for new HCV medicines upon FDA approval of a new drug, estimating total treated patients and list price that were close to actual figures seen in late 2013.[50]

The Competitive Market Is Evolving Rapidly Toward Value-Based Care

Changes in the science and pressures for cost containment in the competitive market are driving rapid evolution of payment and care delivery systems, and biopharmaceutical manufacturers are playing a role in this transformation. As therapies become more personalized, and as the competitive care market moves away from fee-for-service care and toward more integrated care systems, biopharmaceutical companies are increasingly partnering with payers to develop new types of payment arrangements that reward improvements in care and better health outcomes for patients. Recent announcements by a number of manufacturers and health plans illustrate the degree of innovation occurring among organizations in the private sector as do ongoing efforts to evaluate and expand programs like comprehensive medical management and value-based insurance design.

Yet while the science and market are moving rapidly, evolution in payment and contract types has been slowed by regulations designed for an earlier era. Such regulations can have the unintended consequence of making it more difficult for payers to plan ahead, to understand the impact of medicines in avoiding other health costs, and to explore new business models.

For example, current FDA regulations about how manufacturers can communicate about their medicines pre-approval can chill important discussions with payers, since those discussions could be perceived as making claims about the efficacy or safety of an unapproved medicine. Even though payers have a large amount of information about medicines in the research pipeline, they desire greater predictability and certainty as they set their premiums and formularies, often 18 months in advance. Manufacturers have a lot of information that would be useful, such as clarity about the potential scope of the population that may benefit from a new medicine in development. Once a medicine is approved, rules governing what information manufacturers can

[48] Lee LY, Tong CY, et al. New therapies for chronic hepatitis C infection: a systematic review of evidence from clinical trials. Int J Clin Pract. 2012;66(4):342–55. [PubMed]

[49] ECRI Institute. AHRQ Healthcare Horizon Scanning System Potential High-Impact Interventions: Priority Area 09: Infectious Disease Including HIV/AIDS. Rockville, MD: Agency for Healthcare Research and Quality. June 2013. Available at: http://www.effectivehealthcare.ahrq.gov/reports/final.cfm.

[50] Optum. 5 Minutes on Hepatitis C. https://www.optum.com/content/dam/optum/resources/Brief/012214-orc-5min-hep-c.pdf

share should also be updated to assure more rapid dissemination of evidence about the impact of medicines, such as pharmacoeconomic analyses and real world evidence from sources such as anonymized electronic health records. Similarly, government rules meant to deter health care fraud can also result in thwarting beneficial manufacturer programs, such as effective support for medication adherence, which has been demonstrated to improve patient outcomes and reduce use of medical services, with clear benefits for patients.

Additionally, for the competitive market to succeed in driving value for patients, patients and consumers need better access to relevant information about their covered benefits and the impact of their treatment choices on out-of-pocket costs. Employers and other health care stakeholders have recognized that putting quality and cost information in front of patients before they pursue treatment can improve outcomes and lower costs. Greater transparency in these areas can lead to improved patient satisfaction, better health outcomes, and lower total health spending.

The Competitive Market, not Government, Is in the Best Position to Drive Value

The competitive market is uniquely well-designed to make complex determinations about the value of medicines; many, heterogeneous purchasers are best able to assess their own needs in light of the available evidence. In contrast, policies that would impose a centralized government determination of value should be rejected because they would reduce choice, lead to suboptimal outcomes, and undermine continued innovation.

A good case study is the Medicare Part D program. Part D was designed to leverage the competitive market to promote patient access to important medical advances, such as new HCV treatments, while also working to hold down costs. The success of Part D's competitive structure is evidenced by CMS' announcement of relatively stable Part D premiums in 2016, the ability of plans to manage utilization and help keep the benefit affordable for both beneficiaries and taxpayers, and CMS' ability to modify the Part D risk adjustment model to account for novel therapies such as new HCV treatments.

Part D's competitive, market-based structure relies on negotiation between private entities to ensure coverage while driving significant discounting and rebating to help make the program more affordable for all beneficiaries.[51] As seen in the discussion of HCV medicines, private payers and PBMs negotiate discounts and rebates, which help hold down premiums, significantly reduce health plan acquisition costs for covered drugs, and recently led to lower launch prices.

Despite claims that Medicare spending for new and forthcoming HCV treatments would drive up Part D beneficiary premiums and overall program costs, CMS announced last July that estimated average premiums for 2016 will be about $32.50 per month, an amount that has been relatively stable since 2011 and nearly half of the $59 originally projected for 2016. Recent analysis from Milliman found that while program spending trend for HCV treatments in Part D has increased in recent years and is expected to continue in 2016, in the aggregate, manufacturer rebates and

[51] Overview of approaches to control prescription drug spending in federal programs: hearings before the Subcommittee on Federal Workforce, Postal Service, and the District of Columbia, Committee on Oversight and Government Reform, House of Representatives; June 24, 2009. Statement of John E. Dicken, Director, Health Care, Government Accountability Office. http://www.gao.gov/new.items/d09819t.pdf.

contracting improvements will more than offset the increased trend in 2016.[52] Moreover, in 2015, all Part D plans (both stand-alone prescription drug plans and Medicare Advantage prescription drug plans) are covering at least one treatment option, and some treatment options have nearly 100% coverage across all plans. This demonstrates the success of Part D's competitive structure in working to control costs while providing beneficiaries with access to these important treatment options.

Of note, CMS modified the Part D risk adjustment model for the 2016 plan year to account for the introduction of new treatment options for HCV that otherwise would not appear in the historical data used in the model. This adjustment was temporary and will no longer be necessary once these treatment options appear in the historical data. Existing regulatory flexibility such as this is important to reflect the continued introduction of new medicines and utilization changes in the mix of existing drugs, and to ensure that plans are compensated adequately for providing high quality care to beneficiaries.

In contrast, experience in several European countries has shown the dangers of the government attempting to make centralized, one-size-fits-all judgments of value. Restrictions imposed by the United Kingdom's National Institute for Health and Care Excellence (NICE) have created substantial barriers between patients and life-saving treatments – almost 80% of cancer medicines reviewed in the last seven years by NICE have been given some kind of access restriction. Organizations like NICE, which impose centralized value judgments, also create delays to accessing new medicines. Market authorization of new cancer drugs starts later in countries, like the U.K., that use a cost-per-quality adjusted life year mechanism to restrict access, and may never reach the levels seen in other countries without such mechanisms.[53] There should be no mistake that government assessments of value would dramatically expand government's role in deciding which treatments patients do and do not receive.

The current debate over the development and use of value assessment tools by private payers and other organizations in the health care market illustrates that there is a lack of consensus around a single approach to determining the value of medicines. Methods and approaches to value assessment must continue to evolve, and the lack of consensus around an approach serves as an example of why policies that rely on a centralized government value determination are inadvisable.

Notwithstanding the limitations of assessments of medicines currently done in the private sector, the competitive market is far better positioned than government to drive value in ways that are responsive to continued innovation and individual differences. Errors have less sweeping effect and are more easily corrected. New information – particularly important as we move to a rapid learning health care system – is more likely to be incorporated in a timely way through the competitive market. Multiple private sector assessments allow for a diversity of perspectives – which is particularly important since there is wide variability in perspectives on value, both among stakeholders and across patient populations. Research shows the outcomes that matter to patients often are different than those that are a priority for payers or other stakeholders.

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[52] Milliman, "2016 Medicare Part D National Average Value Drivers," Prepared for PhRMA, Oct. 2015.
[53] IMS Institute for Healthcare Informatics. *Impact of cost-per-QALY reimbursement criteria on access to cancer drugs.* December 2014.

Likewise, a centralized government assessment is less likely to recognize the heterogeneity of patients' medical conditions and personal priorities and more likely to become a one-size-fits-all standard, even as new precision medicines make individualization ever more important.

Finally, the importance of avoiding policy based on centralized value assessments was recognized by policymakers in the Affordable Care Act, which includes strong patient protections against the use of cost-effectiveness standards in Medicare. These protections reflect strong public support for good information to help patients and consumers make individualized decisions about value, and equally strong support for protections against centralized government value standards that interfere with individual physician-patient decisions.

Importance of Maintaining Protections for Commercially Sensitive Data

Protections for commercial, confidential information are essential for an effectively functioning health care market. Biopharmaceutical companies already disclose extensive financial information regarding investment in research and development, and operating costs. It is critically important that Congress maintain strong existing protections for commercially sensitive drug cost data (including "data on bids, rebates and other price concessions"),[54] as provided for in the Trade Secrets Act (18 U.S.C. § 1905) and Social Security Act § 1927(b)(3)(D). Disclosure of these sensitive data would undermine the competitive negotiations between health plans and manufacturers, which could result in higher prescription drug costs. This is evident in the findings of the Federal Trade Commission (FTC), which has consistently stated over many years and across different Administrations that requiring disclosure of manufacturer-PBM negotiated price information can "undercut vigorous competition on drug pricing"[55] and "undermine competition … between pharmaceutical manufacturers to offer discounts," which could "raise prescription drug prices for consumers."[56]

Likewise, confidential negotiations between payers and manufacturers have been a key factor in driving lower drug spending growth and are foundational to the success of the Medicare Part D program. CBO has stated, "the revelation of [manufacturers'] rebates to PDPs would create pressure to reduce those rebates, which would tend to increase costs for both the Medicare program and, on average, for enrollees."[57] CBO has estimated the impact of such disclosure as costing the program up to $10 billion over a 10 year period.

Strong protection of commercially sensitive data is fundamental to preserving our competitive market-based system and incentivizing the research and development of innovative new medicines. Therefore, we would oppose any efforts that call for the public release or

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[54] 79 Fed. Reg. 1918, at 1989.

[55] FTC Letter to Terry G. Kilgore, Member, Virginia House of Delegates, re: H.B. 945 (Oct. 2, 2006); FTC Letter to Representative Patrick McHenry, re: North Carolina Bill 1374 (July 15, 2005); FTC Letter to California Assembly Member Greg Aghazarian, re: AB 1960 (Sept. 7, 2004).

[56] FTC Letter to The Honorable Mark Formby, Mississippi House of Representatives, re: SB 2445 (March 22, 2011). See also J. Shepherd, "Is More Information Always Better? Mandatory Disclosure Regulations in the Prescription Drug Market," *Cornell Law Review Online*. 2013; 99(1):1-22: "[N]umerous empirical studies have also established that the disclosure of competitively sensitive information is associated with higher prices. As firms learn of their rivals' cost structures, their willingness to bid aggressively disappears." (citations omitted)

[57] Letter from CBO to Congressman Barton and Congressman McCrery (Mar. 12, 2007).

dissemination of proprietary data, such as confidential data regarding pricing, rebates, and the cost of drug development.

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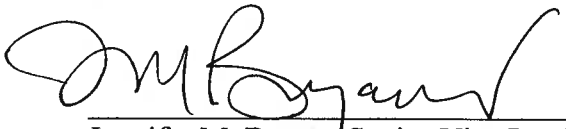
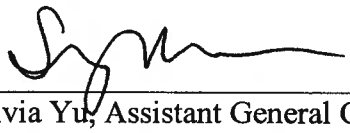
PhRMA believes that the competitive U.S. health care market provides a sound framework for balancing and supporting patient access, cost containment, and continued progress for patients, based on the points outlined above. We would welcome the opportunity to discuss our comments in greater detail.

Sincerely,



Jennifer M. Bryant, Senior Vice President
Policy & Research



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