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**BEFORE THE U.S. SENATE HEALTH, EDUCATION, LABOR AND PENSIONS
COMMITTEE**

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Chairman Alexander, Ranking Member Murray, and Members of the Committee, thank you for inviting me to participate in today's hearing. Understanding the role the drug delivery system plays in determining what patients pay for medicines is a critical part of the discussion about what can be done to improve patient access and affordability and I appreciate the opportunity to explore this topic with you in depth.

PhRMA represents the country's leading innovative biopharmaceutical research companies, which are devoted to discovering and developing medicines that enable patients to live longer, healthier, and more productive lives. The biopharmaceutical sector is one of the most research-intensive industries in the U.S.: since 2000, PhRMA member companies have invested more than half a trillion dollars in the search for new treatments and cures, including \$65.5 billion in 2016 alone.

Medicines Have Transformed the Treatment of Many Diseases, Helping Patients Live Longer and Healthier Lives

We are in a new era of medicine in which breakthrough science is transforming patient care and enabling us to more effectively treat chronic disease, the biggest cost driver in our health care system. Innovative medicines represent significant scientific advancements that revolutionize the treatment and thus the downstream healthcare costs of complex and costly diseases, such as cancer, hepatitis C, HIV/AIDS, and cardiovascular disease. In this new era of medicine, many diseases previously regarded as deadly are now manageable and even curable. Today, more than 7,000 medicines are in development worldwide, of which 80% have the potential to be first in class and 42% are personalized medicines.¹ Prescription medicines produce unparalleled value and savings for the health care system, preventing or slowing the progression of disease, and reducing the need for more intensive medical care. Continued advances in biopharmaceutical innovation represent the best opportunities to improve health outcomes and control future health care costs.

¹ Long G. The Biopharmaceutical Pipeline: Innovative Therapies in Clinical Development. Analysis Group. 2017; Tufts Center for the Study of Drug Development (CSDD). Personalized Medicine Gains Traction But Still Faces Multiple Challenges. Tufts CSDD Impact Report. 2015;17(3).

New medicines help contain overall health care spending 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 by improving the use of medicines.² Similarly, research published 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.³

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.⁴ 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,⁵ and less prevalent conditions such as Parkinson's disease.⁶ More recent research has shown that increased use of medicines among patients is associated with reductions in expenditures from avoided use of inpatient and outpatient services in Medicaid as well. For example, among patients with schizophrenia, improved adherence to antipsychotic medicines yielded annual net savings of up to \$3.3 billion, or \$1,580 per patient per year, driven by lower hospitalizations, outpatient care, and criminal system involvement.⁷ Another study found that if 60% of the children enrolled in Medicaid achieved high adherence to asthma treatment in just 14 states, Medicaid could achieve \$57.5 million in savings annually.⁸

The Competitive Market for Prescription Medicines Balances Innovation, Patient Access, and Cost Containment

The competitive market is the engine that drives the innovative biopharmaceutical research and development ecosystem. 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. Since 2000, biopharmaceutical companies have brought more than 500 new medicines to the U.S. market, resulting in significant progress

² IMS Institute for Healthcare Informatics. Avoidable Costs in U.S. Healthcare: The \$200 Billion Opportunity from Using Medicines More Responsibly. June 2013.

³ Roebuck MC, Lieberman JN, Gemmill-Toyama M, et al. Medication Adherence Leads to Lower Care Use And Costs Despite Increased Drug Spending. *Health Affairs*. 2011;30(1):99.

⁴ Congressional Budget Office. Offsetting Effects of Prescription Drug Use on Medicare's Spending for Medical Services. November 29, 2012.

⁵ Roebuck MC. Medical Cost Offsets from Prescription Drug Utilization Among Medicare Beneficiaries. *Journal of Managed Care Pharmacy*. 2014;20(10):994-995.

⁶ Wei YJ, Palumbo FB, Simoni-Wastila L, et al. Antiparkinson Drug Adherence and its Association With Health Care Utilization and Economic Outcomes in a Medicare Part D Population. *Value in Health*. 2014;17(2):196-204.

⁷ Predmore ZS, Mattke S, Horvitz-Lennon M. Improving Antipsychotic Adherence among Patients With Schizophrenia: Savings for States. *Psychiatric Services*. 2015; 66:343-345.

⁸ Rust G, Zhang S, McRoy L. Potential Savings From Increasing Adherence to Inhaled Corticosteroid Therapy in Medicaid-Enrolled Children. *American Journal of Managed Care*. 2015;21(3):173-180.

against some of the most costly and challenging diseases.⁹ Yet, as a result of robust negotiation and competition in the marketplace, spending on medicines is growing at the slowest rate in years.¹⁰

Government, market analyst, and pharmacy benefit manager data all point to the same conclusion: that after peaking in 2014—an anomaly year in which millions of uninsured patients gained coverage and a record number of new medicines were approved—prescription drug spending growth has fallen substantially. Accounting for discounts and rebates, multiple sources report that spending on prescription medicines grew by just 3% to 5% in 2016.¹¹ As a result of negotiation and competition in the marketplace, spending on retail and physician-administered medicines continues to represent only 14% of overall health care spending, even though scores of new medicines are approved every year. And at the state level, Medicaid programs spent just 4.9% of their budgets on prescription drugs, including new medicines, in 2016, relative to 26% for hospital care and 18.2% for provider services.¹²

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.¹³ 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.¹⁴ Meanwhile, the average charge for percutaneous transluminal coronary angioplasty (PTCA) – a surgical procedure to treat cardiovascular disease – increased by almost 66% during that same time period.¹⁵

⁹ 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>; US Food and Drug Administration. New Drugs at FDA: CDER's New Molecular Entities and New Therapeutic Biological Products. 2012 – 2015.

<https://www.fda.gov/drugs/developmentapprovalprocess/druginnovation/default.htm>

¹⁰ QuintilesIMS Institute. Medicine Use and Spending in the US: A Review of 2016 and Outlook to 2021. April 2017

¹¹ QuintilesIMS Institute. Medicine Use and Spending in the US: A Review of 2016 and Outlook to 2021. April 2017; CVS Health. CVS Health PBM Clients Achieved Lowest Prescription Drug Trend in Four Years, Despite Rising Drug Prices. March 15, 2017. <http://www.prnewswire.com/news-releases/cvs-health-pbm-clients-achieved-lowest-prescription-drug-trend-in-four-years-despite-rising-drug-prices-300423726.html>; Express Scripts. 2016 Drug Trend Report. February 2017. <https://lab.express-scripts.com/lab/drug-trend-report>; Which PBM Best Managed Drug Spending in 2016: How Did OptumRx Compare? Drug Channels. April 25, 2017. <http://www.drugchannels.net/2017/04/which-pbm-best-managed-drug-spending-in.html#more>.

¹² Prescription drug pre-rebate expenditures tabulated by The Menges Group using FY2016 CMS State Drug Utilization data files and CMS brand/generic indicators for each National Drug Code. Rebate information obtained from FY2016 CMS-64 reports. Post-rebate expenditures derived through The Menges Group tabulations using above information.

¹³ IMS Institute for Healthcare Informatics. Price Declines After Branded Medicines Lose Exclusivity in the US January 2016.

¹⁴ 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).

¹⁵ Data adapted from: HCUP Hospital Charge Database 2005 to 2013, Average Hospital Charges.

The U.S. market is structured to take maximum advantage of savings from brand competition and from generics. Three large, sophisticated pharmacy benefit managers (PBMs) manage over 75% of all prescriptions filled.¹⁶ They use brand competition to obtain discounts from manufacturers and take full advantage of the presence of generics to drive savings. 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. As one example of the growing influence of PBMs, industry leader Express Scripts has publicly stated their success in leveraging substantial rebates for hepatitis C medicines led to those treatments being less expensive in the U.S. than in many other western countries.¹⁷ And the competitive market will continue to generate savings in the years ahead, as more than \$140 billion of U.S. brand sales are projected to face generic competition between now and 2021.¹⁸ Competition from biosimilars is estimated to account for \$38 billion of the loss in brand spending.

List Prices for Medicines Do Not Reflect Substantial Rebates and Discounts and Provide an Increasingly Inaccurate Picture of Prescription Drug Costs

Much of the public debate about the cost of medicines has focused on list prices, which do not account for the rebates and discounts that PBMs and health plans commonly negotiate with biopharmaceutical companies in exchange for preferred formulary placement on lower cost-sharing tiers. For certain medicines used to treat chronic conditions like asthma, high cholesterol, hepatitis C, and diabetes, these discounts and rebates can reduce list prices by as much as 30% to 70%.¹⁹ Biopharmaceutical companies are also required to provide sizable statutory rebates, discounts, and fees to government programs, which have increased in recent years due to an increase in the Medicaid rebate, closing of the Medicare Part D “donut hole” and

¹⁶ Fein AJ; Pembroke Consulting, Inc., and Drug Channels Institute. 2014-15 Economic Report on Retail, Mail, and Specialty Pharmacies. January 2015. <http://drugchannelsinstitute.com/files/2014-15-PharmacyIndustry-Overview.pdf>

¹⁷ 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>

¹⁸ QuintilesIMS Institute. Medicine Use and Spending in the US: A Review of 2016 and Outlook to 2021. April 2017.

¹⁹ QuintilesIMS Institute. Estimate of Medicare Part D Costs After Accounting for Manufacturer Rebates. October 2016; Gronholt-Pedersen J, Skydsgaard N, Neely J. Novo Nordisk Defends U.S. Diabetes Drug Pricing. *Reuters*. November 4, 2016. <http://www.reuters.com/article/us-novo-nordisk-prices-idUSKBN12Z184>; Silverman E. What the ‘Shocking’ Gilead Discounts on its Hepatitis C Drugs Will Mean. *Wall Street Journal*. February 4, 2015. <https://blogs.wsj.com/pharmalot/2015/02/04/what-the-shocking-gilead-discounts-on-its-hepatitis-c-drugs-will-mean/> Barrett P, Langreth R. The Crazy Math Behind Drug Prices: Intermediaries that Negotiate to Lower Prices May Cause Them To Increase Too. *Bloomberg Businessweek*, June 29, 2017. <https://www.bloomberg.com/news/articles/2017-06-29/the-crazy-math-behind-drug-prices>

expansion of the 340B program. These mandatory payments grew by more than 40% between 2013 and 2015, increasing from \$29.6 billion to \$41.8 billion.²⁰

Excluding rebates and discounts from discussions about the cost of prescription medicines provides an increasingly inaccurate picture of marketplace trends. According to PBMs and industry analysts, list prices for brand medicines have grown by an estimated 9% to 12% annually since 2015, while net prices (which take discounts and rebates into account) have grown by just 2.5% to 3.5%.²¹ A recent study from the QuintilesIMS Institute demonstrates that net prices for medicines that have been on the market for at least two years *declined* by an average of 2.5% annually from 2010 to 2016, driven by patent expirations and increased competition from generics.²² The QuintilesIMS report also notes that over the next five years, net prices for existing medicines will continue to decline between 1% and 4% annually, highlighting the important role rebates and discounts will continue to play in containing prescription medicine spending growth in the future.

Claims from PBMs, payers, and others about the skyrocketing prices of medicines almost always focus solely on list prices, which are not reflective of actual spending trends. When new hepatitis C medicines offering cure rates exceeding 90% entered the market, PBMs claimed that these life-saving treatments and cures would bankrupt the health system and their costs were simply unsustainable. Instead, competition among brand manufacturers quickly drove deep discounts averaging 40% to 65% off the list price.²³ Express Scripts now states that their aggressive negotiations have saved Americans \$4 billion, cured more patients with hepatitis C than any time in history, and that the discounted price makes it affordable to treat all patients with the infection.²⁴

Prior to the launch of 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, projecting up to \$150 billion to \$200 billion per year in spending for these medicines.²⁵ CMS' Office of the Actuary, however, projected a much more modest impact,

²⁰ Berkeley Research Group. The Pharmaceutical Supply Chain: Gross Drug Expenditures Realized by Stakeholder. January 2017.

²¹ QuintilesIMS Institute. Medicine Use and Spending in the US: A Review of 2016 and Outlook to 2021. April 2017; Express Scripts. 2016 Drug Trend Report. February 2017. <https://lab.express-scripts.com/lab/drug-trend-report>; SSR Health. US Brand Pharmaceutical Net Prices Fell 0.3% in 3Q16. January 18, 2017. <http://www.ssrlc.com/publication/us-brand-pharmaceutical-net-prices-fell-0-3-in-3q16/>

²² QuintilesIMS Institute. Understanding the Drives of Drug Expenditure in the US. September 2017.

²³ What Gilead's Big Hepatitis C Discounts Mean for Biosimilar Pricing. Drug Channels. February 5, 2015. <http://www.drugchannels.net/2015/02/what-gileads-big-hepatitis-c-discounts.html>

²⁴ 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>

²⁵ 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/>

based on expected competition leading to discounts and continued widespread use of generic statins.²⁶ The Actuary's refusal to accept these inflated claims proved to be the right approach. In fact, PBMs quickly made deals to cover both of the brand competitors on the market and emphasized that the drugs' cost is "far lower than industry forecasts."²⁷ New research shows that PBMs have also effectively used strict prior authorization and high cost-sharing requirements to suppress utilization of these medicines, resulting in less than one-third of patients prescribed a PCSK9 inhibitor being able to access therapy.²⁸

A Complex Distribution and Payment System Shapes the Prices Patients, Health Plans, and the Government Pay for Medicines

The process by which prescription medicines move from biopharmaceutical manufacturers to patients involves multiple stakeholders and numerous financial transactions. This process has evolved significantly in recent years, as supply chain entities have grown to play a larger role in drug distribution and payment. Wholesalers, pharmacies, plan sponsors, and patients all pay different prices for medicines, and the amount that is ultimately paid is determined by confidential negotiations between stakeholders. Many discounts provided by manufacturers do not flow directly through to the patients taking the medicine, and in some cases the full discounts may also not flow through to employers or plan sponsors.²⁹

Some manufacturer rebates and discounts are required by law, while others are negotiated between biopharmaceutical companies and powerful commercial payers, many of which cover tens of millions of patients. In recent years, as payers have consolidated and competition between brand medicines has increased, negotiated rebates and discounts have also grown. Multiple data sources indicate that growth in manufacturer rebates and discounts has been substantial and that an increasing share of these discounts and rebates are retained by middlemen involved in distributing and paying for prescription medicines.³⁰ According to a recent study by the Berkeley Research Group, on average, more than a third of the initial list price of a medicine is rebated back to insurance companies, PBMs and the government, or retained by other

²⁶ Kelly C. U.S. Drug Spending Will Increase 7.6% in 2015, Including PCSK9 Costs – CMS. *The Pink Sheet*, July 2015.

²⁷ 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>

²⁸ Navar AM, Taylor B, Mulder H, et al. Association of Prior Authorization and Out-of-Pocket Costs With Patient Access to PCSK9 Inhibitor Therapy. *JAMA Cardiology*. Published online September 27, 2017. doi:10.1001/jamacardio.2017.3451.

²⁹ Midwestern Business Group on Health. Drawing a Line in the Sand: Employers Must Rethink Pharmacy Benefit Strategies. September 2017. https://higherlogicdownload.s3.amazonaws.com/MBGH/4f7f512a-e946-4060-9575-b27c65545cb8/UploadedImages/Specialty%20Pharmacy/DMJ_MBGH_Line_in_the_Sand_RV12_9617.pdf

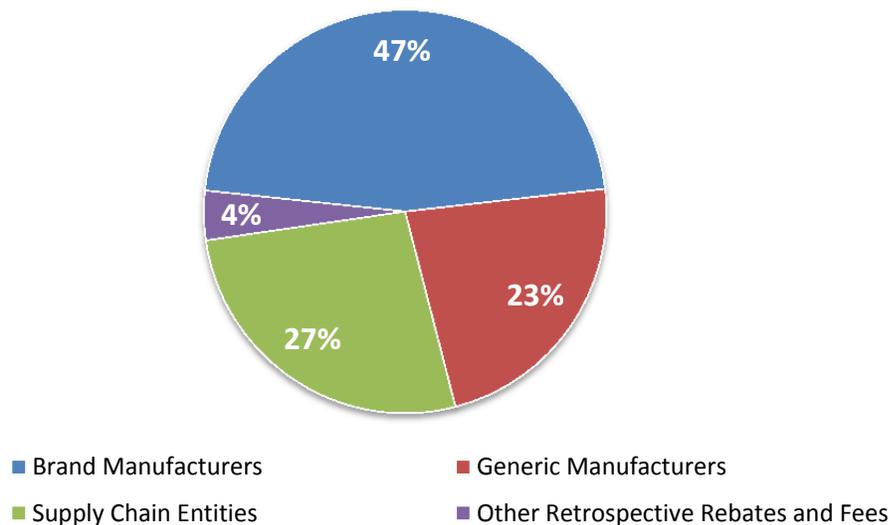
³⁰ QuintilesIMS Institute. Medicine Use and Spending in the US: A Review of 2016 and Outlook to 2021. April 2017; Berkeley Research Group. The Pharmaceutical Supply Chain: Gross Drug Expenditures Realized by Stakeholder. January 2017; Dross D. Will Point-of-Sale Rebates Disrupt the PBM Business? Mercer. July 31, 2017. <https://www.mercer.us/our-thinking/healthcare/will-point-of-sale-rebates-disrupt-the-pbm-business.html>

stakeholders along the biopharmaceutical supply chain.³¹ And the gap between list prices and net prices is growing every year as more of medicine costs are being retained by middlemen in the system.

As shown in Figure 1, accounting for the discounts, rebates and fees paid to PBMs, payers, and the government, brand biopharmaceutical companies realize less than half of total net spending on prescription medicines.³² Of the \$469 billion spent on prescription drugs in the U.S. in 2015, brand manufacturers realized \$219 billion; the remainder went to generic manufacturers or was retained as earnings by entities along the supply chain and other stakeholders.³³ The \$219 billion realized by the brand biopharmaceutical industry accounts for just 6.8% of the \$3.2 trillion spent on health care overall in the U.S. in 2015.³⁴

Figure 1:

Share of 2015 Net Prescription Medicine Spending Realized by Manufacturer and Non-Manufacturer Stakeholders



Patients Do Not Directly Benefit from Significant Price Negotiations Happening in the Market Today

Savings generated from price negotiations between biopharmaceutical companies and payers do not always make their way directly to patients facing high cost-sharing for their medicines. Unlike care received at an in-network hospital or physician's office, health plans base cost-

³¹ Berkeley Research Group. The Pharmaceutical Supply Chain: Gross Drug Expenditures Realized by Stakeholder. January 2017.

³² *Ibid.*

³³ *Ibid.*

³⁴ Martin AB, Hartman M, Washington B, et al. National Health Spending: Faster Growth in 2015 As Coverage Expands and Utilization Increases. *Health Affairs*. 2017;36(1):166-176.

sharing for prescriptions filled in the deductible or with coinsurance on undiscounted list prices, rather than on prices that reflect negotiated rebates and discounts. Enrollment in high deductible health plans and use of coinsurance for prescription medicines has grown sharply in recent years, increasingly exposing patients to high out-of-pocket costs based on undiscounted prices, creating scenarios in which medicines appear to be more costly than other health care services. High cost-sharing is a cause for concern, as a substantial body of research clearly demonstrates that increases in out-of-pocket costs are associated with both lower medication adherence and increased abandonment rates, putting patients' ability to stay on needed therapies at risk.³⁵

Over the past 10 years, patient cost-sharing has risen substantially faster than health plan costs. For workers with employer-sponsored health insurance, out-of-pocket spending for deductible and coinsurance payments increased by 230% and 89%, respectively, compared to a 56% increase in payments by health plans.³⁶ Whereas cost-sharing for prescription medicines once consisted almost entirely of copays, use of deductibles and coinsurance has increased rapidly. For example, the share of patient out-of-pocket drug spending represented by coinsurance more than doubled over the past ten years in the commercial market, while the share accounted for by deductibles tripled.³⁷ The growing use of deductibles and coinsurance for prescription medicines creates affordability challenges for many patients. Patients enrolled in high deductible health plans may be asked to pay thousands of dollars out-of-pocket before any of their prescriptions are covered, while patients with coinsurance are responsible for as much as 30% to 40% of the total cost of their medicines.

Due to the growing gap between list and net prices, patients' cost sharing for medicines is increasingly based on prices that do not reflect plan sponsors' actual costs. For example, market analysts report that negotiated discounts and rebates can lower the net price of insulin by up to 50% to 70%, yet health plans require patients with deductibles to pay the full undiscounted price. As a result, a patient in a high-deductible health plan who pays the list price each month for insulin maybe paying hundreds—or even thousands—more annually than their insurer. Analysis by Amundsen Consulting shows that more than half of patients' out-of-pocket spending for

³⁵ IMS Institute for Healthcare Informatics. Emergency and Impact of Pharmacy Deductibles: Implications for Patients in Commercial Health Plans. September 2015; Doshi JA, Li P, Huo H, et al. High Cost Sharing and Specialty Drug Initiation Under Medicare Part D: A Case Study in Patients with Newly Diagnosed Chronic Myeloid Leukemia. *American Journal of Managed Care*. 2016;22(4 Suppl):S78-S86; Brot-Goldberg ZC, Chandra A, Handel BR, et al. What Does A Deductible Do? The Impact of Cost-Sharing on Health Care Prices, Quantities, and Spending Dynamics. NBER Working Paper 21632, October 2015; Eaddy MT, Cook CL, O'Day K, et al. How Patient Cost-Sharing Trends Affect Adherence and Outcomes. *Pharmacy & Therapeutics*. 2012;37(1):45-55.

³⁶ Claxton G, Levitt L, Long M, et al. Increases in Cost-Sharing Payments Have Far Outpaced Wage Growth. Peterson-Kaiser Health System Tracker. October 4, 2017. <https://www.healthsystemtracker.org/brief/increases-in-cost-sharing-payments-have-far-outpaced-wage-growth/#item-start>

³⁷ Claxton G, Levitt L, Long M. Payments for Cost Sharing Increasing Rapidly Over Time. Peterson-Kaiser Health System Tracker. April 2016. <http://www.healthsystemtracker.org/insight/examining-high-prescription-drug-spending-for-people-with-employer-sponsored-health-insurance/>

brand medicines is based on the list price of the medicine, even though their health insurer may be receiving a steep discount.³⁸

Health plans typically use some portion of negotiated rebates to reduce premiums for all enrollees, rather than to directly lower costs for patients facing high cost-sharing due to deductibles and coinsurance. According to one actuarial firm, this results in a system of “reverse insurance,” whereby payers require patients with high drug expenditures to pay more out-of-pocket, while rebate savings are spread out among all health plan enrollees in the form of lower premiums.³⁹ Asking sicker patients with high drug costs to subsidize premiums for healthier enrollees is the exact opposite of how health insurance is supposed to work.

Some patients also end up paying more at the pharmacy counter when they use their insurance, not knowing that their prescriptions would be cheaper if they were paying in cash. Many PBM contracts require pharmacies to charge patients the exact amount negotiated between the PBM and the pharmacy, even if that amount exceeds what the pharmacy would charge to a patient without insurance. Gag-clauses in PBM contracts prohibit pharmacists from informing insured patients about the lower cash price, at the risk of the pharmacy being excluded from the PBM’s network. In these instances, pharmacies must instead overcharge patients, requiring them to pay the full amount of their copayment, over and above the actual cost of the medication. These overpayments are then “clawed back” from the pharmacy by the PBM.⁴⁰

PBMs Negotiate Lower Medicine Prices for Health Plans and Employers, But Don’t Always Pass Along All of the Savings

PBMs commonly retain a portion of the rebates they negotiate on behalf of their health plan and employer clients. While the remainder of the rebates are generally passed on to plan sponsors, smaller employers and health plans may not benefit from all of the price concessions the PBM has negotiated with manufacturers, particularly if the PBM decides not to define certain fees or other concessions as “rebates.” For example, one benefits consultant has observed that PBMs are increasingly changing the contractual definition of rebates to exclude certain administrative fees, allowing the PBM to retain these payments rather than passing them back to the plan

³⁸ Amundsen Consulting. Commercially-Insured Patients Pay Undiscounted List Prices for One In Five Brand Prescriptions, Accounting for Half of Out-of-Pocket Spending on Brand Medicines. March 2017. <http://www.phrma.org/report/commercially-insured-patients-pay-undiscounted-list-prices-for-one-in-five-brand-prescriptions-accounting-for-half-of-out-of-pocket-spending-brand-medicines>

³⁹ Girod CS, Hart SK, Wertz S. 2017 Milliman Medical Index. May 2017. <http://www.milliman.com/uploadedFiles/insight/Periodicals/mmi/2017-milliman-medical-index.pdf>

⁴⁰ Hopkins JS. You’re Overpaying for Drugs and Your Pharmacist Can’t Tell You. *Bloomberg*. February 24, 2017. <https://www.bloomberg.com/news/articles/2017-02-24/sworn-to-secrecy-drugstores-stay-silent-as-customers-overpay>

sponsor. These administrative fees can be as high as 25% to 30% of the total rebate negotiated with the manufacturer and are often not reported to the plan sponsor by the PBM.⁴¹

In addition to the rebates they negotiate with biopharmaceutical companies, PBMs are increasingly requiring that if a medicine's list price increases by more than a certain percentage, the manufacturer must provide an additional price protection rebate reimbursing the PBM for all price increases above the threshold. Lack of transparency in contracts between employers and PBMs has led many plan sponsors to question the share of rebate savings being passed through, how much the PBM is retaining for administrative fees, and whether the PBM is disclosing and passing on other price concessions, such as savings from price protection rebates.⁴²

Both the portion of the rebate retained by the PBM and the administrative fees they charge their clients are typically based off of a percentage of a medicine's list price. Accordingly, some PBMs may prefer that their formularies include medicines with high list prices and large rebates, rather than medicines with a lower list price. In its most recent report to Congress, the Medicare Payment Advisory Commission discussed incentives that may drive Part D plan sponsors to give formulary preference to medicines with large rebates, rather than lower cost alternatives.⁴³ These incentives arise because sizable portions of the Part D benefit are not paid for by plan sponsors (e.g., beneficiaries and manufacturers pay for the majority of costs in the coverage gap). Similarly, the Centers for Medicare & Medicaid (CMS) has noted that coverage of medicines with high list prices and large rebates “ease[s] the financial burden borne by Part D plans essentially by shifting costs to the catastrophic phase of the benefit, where plan liability is limited.”⁴⁴

Hospital Markups on Medicines Increase Cost-Sharing for Commercially-Insured Patients

The pharmaceutical distribution and payment process differs for medicines administered in a physician office or health care facility vs. those purchased at a pharmacy. Providers typically purchase medicines directly, often through a Group Purchasing Organization (GPO). After the physician administers the medicine to the patient, the patient's insurance reimburses the provider for the cost of the medicine as part of the patient's coverage for medical care.

⁴¹ Dross D. Will Point-of-Sale Rebates Disrupt the PBM Business? Mercer. July 31, 2017.

<https://www.mercer.us/our-thinking/healthcare/will-point-of-sale-rebates-disrupt-the-pbm-business.html>

⁴² Midwestern Business Group on Health. Drawing a Line in the Sand: Employers Must Rethink Pharmacy Benefit Strategies. September 2017. https://higherlogicdownload.s3.amazonaws.com/MBGH/4f7f512a-e946-4060-9575-b27c65545cb8/UploadedImages/Specialty%20Pharmacy/DMJ_MBGH_Line_in_the_Sand_RV12_9617.pdf

⁴³ Medicare Payment Advisory Commission. Status Report on the Medicare Prescription Drug Program (Part D). March 2017. http://medpac.gov/docs/default-source/reports/mar17_entirereport.pdf

⁴⁴ Centers for Medicare & Medicaid. Medicare Part D—Direct and Indirect Remuneration (DIR). January 19, 2017. <https://www.cms.gov/Newsroom/MediaReleaseDatabase/Fact-sheets/2017-Fact-Sheet-items/2017-01-19-2.html>

The amount that providers charge for medicines and how much insurers pay varies widely based on where the medicine is administered to the patient. For example, commercial insurers often pay hospital outpatient departments twice as much as physician offices for administering the exact same medicines, including for diseases such as cancer or autoimmune disorders.⁴⁵ This is because large hospitals can demand much higher prices from commercial insurers than small physician practices. The Senior Vice President of Oncology and Genetics at UnitedHealthcare described the effect for chemotherapy treatment at high profile cancer centers: “Put simply, the hospitals are saying, ‘If you want our beds, you have to take our prices for oncology treatment.’”⁴⁶

The results of hospital markups are astounding. Recent research shows that for twenty medicines administered in hospital outpatient departments, hospitals charge prices that are on average nearly five times higher than their acquisition costs and are reimbursed up to three and a half times their acquisition cost by commercial insurers.⁴⁷ For a vast majority of the medicines included in the analysis, this means that the manufacturer—who made the substantial time and R&D investments including clinical trials necessary to develop the treatment—was paid less for the medicine than the hospital.

Hospital markups on prescription medicines have a substantial effect not just on overall healthcare costs, but also on patient affordability. For patients with commercial insurance, coinsurance is the most common form of cost-sharing for provider-administered medicines, which means that the amount the patient must pay is equal to a percentage of the total price the insurer reimburses the provider for the medication. So, when a hospital is paid two or three times the acquisition cost for a medicine, patients are also paying higher coinsurance. As the same United insurance executive quoted above noted “it is immoral to force vulnerable patients to pay triple-digit mark-ups because they have cancer.”⁴⁸

Market Distortions Created by the 340B Program Lead to Higher Health Care Costs

The 340B program, a program originally intended to provide discounts on medicines for safety-net providers, is contributing to higher health care costs and economists suspect that it is also leading to higher list prices for medicines. This program started in 1992, and its basic structure has not been updated since then, despite dramatic changes in the health care system over the past 25 years. The current structure of the program is causing higher health care costs for three main reasons.

⁴⁵ Magellan RX Management. Medical Pharmacy Trend Report: 2016 Seventh Edition. March 2017.

⁴⁶ Newcomer LN. Those Who Pay Have A Say: A View On Oncology Drug Pricing and Reimbursement. *The Oncologist*. 2016;21(7):779-81.

⁴⁷ The Moran Company. Hospital Charges and Reimbursement for Drugs: Analysis of Markups Relative to Acquisition Cost. October 2017.

⁴⁸ Newcomer LN. Those Who Pay Have A Say: A View On Oncology Drug Pricing and Reimbursement. *The Oncologist*. 2016;21(7):779-81.

First, the 340B discount, which is structured as a percentage discount, creates incentives for hospitals to earn a larger spread from the 340B discounts by prescribing more medicines and higher cost medicines. Economists have noted this may lead prescribing to “shift toward more expensive drugs because profit margins will in general be larger.”⁴⁹ A 2015 Government Accountability Office study found evidence that 340B was leading to the prescribing of more drugs and more expensive drugs for Medicare patients.⁵⁰

Second, evidence suggests the 340B program shifts care to more expensive and less convenient settings. Government reports suggest that hospitals are taking advantage of guidance that has not been revisited since 1994 which allows hospitals to obtain more 340B discounts by buying community-based physician practices, so that prescriptions written by those physicians then qualify for 340B discounts.⁵¹ As a result, patients are left with fewer community-based provider options and are pushed into higher cost hospital-based settings. Analysis by the IMS Institute for Healthcare Informatics found that average costs for administering cancer drugs are typically twice as high at hospital outpatient departments compared to community-based oncologists, which can lead to “higher patient cost responsibility.”⁵² Researchers from Memorial Sloan Kettering have noted 340B is helping to drive consolidation of physician practices into hospitals, and that in the absence of reforms “the trend toward consolidation will continue to drive up the cost of commercial insurance...”⁵³

Third, the scale of the program as well as its rapid growth may be affecting market prices for prescription drugs. In 2015, roughly 45 percent of all hospitals participated in 340B.⁵⁴ In an analysis of prescription drug pricing published in the *New England Journal of Medicine*, economists at Harvard University and the University of Chicago concluded that “lawmakers could lower the price of prescription drugs by reforming the federal 340B Drug Pricing Program. [...]The scope of the 340B program is currently so vast for drugs that are commonly infused or injected into patients by physicians that their prices are probably driven up for all consumers” (emphasis added).⁵⁵ Another study in *JAMA* noted that list prices for drugs are likely higher than they otherwise would be “to offset revenue losses incurred as a larger number of drug sales

⁴⁹ Conti R, Bach P. Cost Consequences of the 340B Drug Discount Program. *Journal of the American Medical Association*. 2013;309(19):1995-1996.

⁵⁰ Government Accountability Office. Medicare Part B Drugs: Action Needed to Reduce Financial Incentives to Prescribe 340B Drugs at Participating Hospitals. GAO-15-442, June 2015.

⁵¹ 59 Federal Register 47884.

⁵² IMS Institute for Healthcare Informatics. Global Oncology Trend Report: A Review of 2015 and Outlook to 2020. June 2016.

⁵³ Bach P and Jain RH. Physician’s Office and Hospital Outpatient Setting in Oncology: It’s About Prices, Not Use. *Journal of Oncology Practice* 2017; 13(1), 4-5.

⁵⁴ Medicare Payment Advisory Commission. Report to the Congress: Overview of the 340B Drug Pricing Program. May 2015.

⁵⁵ Conti R and Rosenthal M. Pharmaceutical Policy Reform—Balancing Affordability with Incentives for Innovation. *New England Journal of Medicine*. 2016; 374:703-706.

become eligible for 340B discounts (and thus fewer drugs are sold at full price).”⁵⁶ Certain drug classes are disproportionately impacted by the 340B program. Thus, the price distorting impact may be concentrated in certain therapeutic areas, such as medicines for cancer. For example, sales to 340B hospitals account for 33% of *all* Medicare Part B reimbursement for certain types of cancer drugs.⁵⁷

Market-Based Approaches Are the Best Solution for Addressing Health Care Affordability and Controlling Costs

The competitive U.S. health care market provides a sound framework for balancing and supporting patient access, cost containment, and continued progress for patients. Meaningful efforts to address the cost of prescription medicines must include all stakeholders in the supply chain, including biopharmaceutical companies, PBMs, health plans, wholesalers, hospitals, and pharmacies. Policies targeted solely at brand manufacturers—which account for just half of total net spending on prescription medicines and just 6.8% of total U.S. health care spending—are insufficient for addressing broader health care sustainability challenges and risk diminishing the incentives for future innovation.

Strategies for strengthening and enhancing the competitive market include encouraging payers to share negotiated savings with patients at the pharmacy; reforming outdated regulations hindering the adoption of value-based payment arrangements; reforming the 340B drug discount program, which is distorting the market, so that it better serves the purpose for which it was created; and continuing to modernize the Food and Drug Administration (FDA) and assure that there is robust generic and biosimilar competition once a brand medicine loses its exclusivity.

Sharing Negotiated Savings with Patients

Changes in insurance coverage for prescription medicines, and the growing use of deductibles and coinsurance in particular, have created affordability challenges for many patients. Health plans should be encouraged to directly pass on more of the savings from negotiated rebates in the form of lower patient out-of-pocket costs, just like they do for other types of health care services. This should be executed in a way that maintains the confidentiality of proprietary pricing information that the Federal Trade Commission has identified as important to the effective functioning of competitive markets. Payers have begun to recognize that using the undiscounted price of a medicine to set cost-sharing is problematic for patients: recent statements from the two largest PBMs note that high deductibles for medicines put patients in a “very difficult

⁵⁶Conti R, Bach P. Cost Consequences of the 340B Drug Discount Program, *Journal of the American Medical Association*. 2013;309(19):1995-1996. .

⁵⁷ Drugs sold to 340B hospitals account for 33% of *all* Part B reimbursement for breast cancer and multiple myeloma drugs. Vandervele A and Blalock E. Measuring the Relative Size of the 340B Program: 2012-2017. Berkeley Research Group, July 2017.

position” and indicate that sharing rebate savings directly with patients should be considered as a “best practice.”⁵⁸ Actuarial research indicates that sharing negotiated savings could save certain commercially insured patients enrolled in plans with high deductibles and coinsurance between \$145 and \$800 annually, while increasing premiums by 1% or less.⁵⁹

To help patients afford their medicines, biopharmaceutical companies have entered into partnerships with third parties, such as Blink Health and GoodRx, to offer discounted prices directly to patients, outside of their insurance benefit.⁶⁰ Encouraging health plans to allow the cost of prescriptions purchased through these third-party programs to count towards patients’ deductibles and maximum out-of-pocket spending limits would further reduce patient affordability barriers.

Copay assistance programs offered by biopharmaceutical companies provide another valuable source of assistance for many commercially insured patients who are struggling to afford their out-of-pocket costs, as do manufacturer-sponsored patient assistance programs that help underinsured and uninsured patients obtain the medicines they need for free or nearly free. Recent efforts by health plans to restrict use of copay assistance programs, including no longer counting the full amount patients are asked to pay out-of-pocket towards their deductibles or out-of-pocket maximums, unfairly penalize patients and threaten their ability to stay on needed medicines.

Reforming Outdated Regulations Hindering the Adoption of Value-Based Payment Arrangements

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 companies are playing a role in this transformation. As therapies become more personalized, and as the health 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.

Yet while the science and market are moving rapidly, efforts to develop new ways to pay for medicines have been slowed by regulations designed for an earlier era. Such regulations can have the unintended consequence of making it more difficult for payers to prioritize results that

⁵⁸ Seeking Alpha. Express Scripts Holding (ESRX) Q4 2016 Results – Earnings Call Transcript. February 15, 2017. <http://seekingalpha.com/article/4046365-express-scripts-holding-esrx-q4-2016-results-earnings-call-transcript>; Seeking Alpha. CVS Health (CVS) Q4 2016 Results – Earnings Call Transcript. February 9, 2017. <http://seekingalpha.com/article/4044425-cvs-health-cvs-q4-2016-results-earnings-call-transcript?part=single>

⁵⁹ Bunger A, Gomberg J, Petroske J. Sharing Rebates May Lower Patient Costs and Likely Has Minimal Impact on Premiums. October 12, 2017. <http://www.phrma.org/report/point-of-sale-rebate-analysis-in-the-commercial-market>

⁶⁰ Thomas K. New Online Tools Offer Path to Lower Drug Prices. *New York Times*. February 9, 2016. <https://www.nytimes.com/2016/02/10/business/taming-drug-prices-by-pulling-back-the-curtain-online.html>

matter to patients, and for biopharmaceutical companies to increase the amount of risk they share with payers. For example:

- Ambiguity in FDA rules governing manufacturer communications about their medicines can prevent biopharmaceutical companies from entering into contracts based on the ability of their medicine to reduce hospitalizations or other medical services, since those contracts might be perceived as promoting the medicines for an unapproved indication.
- Lack of clarity in the anti-kickback statute (AKS) can inhibit value-based contracts due to lack of certainty as to whether contracts fit within existing safe harbors and exceptions. By revising the AKS regulations to add a value-based contracting safe harbor, policymakers can facilitate private payers and manufacturers to expand the use of value-based contracts as a solution to health care affordability and controlling drug costs.
- Price reporting rules such as Medicaid Best Price can limit the amount of risk biopharmaceutical companies share with payers within a value based arrangement, because any increased discount beyond the statutory minimum must be offered not only to that payer, but also to all of Medicaid. Exempting value-based arrangements from existing technical and complex Best Price, Average Manufacturer Price, and potentially Average Sales Price requirements to reflect a modern and flexible approach to price reporting would foster expansion of innovative contracting arrangements.

Modernizing the FDA

As the pace of scientific discovery accelerates, it is critical to assure that our regulatory infrastructure keeps up with the science and that FDA regulations are up-to-date, practical, clear and not overly burdensome to foster efficiency, predictability, and the ability of biopharmaceutical companies to innovate and bring new medicines to patients. The Committee's recent action to reauthorize the Prescription Drug User Fee Act creates a solid foundation not only to accelerate approval of new life-saving treatments, but also assure that there is robust generic and biosimilar competition. We thank the Committee for its rapid and bipartisan action.

Accelerating the introduction of new medicines allows the forces of private market competition to keep costs in check and increases the number lifesaving drugs becoming available to patients. Importantly, key provisions of the prescription drug, biosimilar, and generic drug user fee acts will help to eliminate the generic drug application backlog, increase resources to prevent future backlogs, and to streamline the review process and enhance FDA's expertise related to drug-device combination products, an area in which regulatory uncertainties and delays have previously deterred brand and generic manufacturers from investments. Additional opportunities to improve competition include finalizing FDA guidances related to biosimilars and enhancing incentives for generic manufacturers to enter the marketplace where there are no

intellectual property or regulatory incentives preventing generic entry but, due to small patient population sizes, there are no brand or generic competitors. Increased competition from generics could be spurred by waiving user fees for eligible products, providing a transferable generic drug priority review voucher, and expediting review of such products and the inspection of their facilities.

Finally, the FDA can further spur efficiency in the market and free up scarce resources through elimination of certain outdated regulations. For example, regulations requiring biopharmaceutical companies to submit postmarketing reports in a format unique to the U.S. are inefficient and burdensome and provide no appreciable benefit compared to the format used globally. A more logical approach for submission of postmarketing reports would be to streamline the formats. Similarly, requiring biopharmaceutical companies to submit all promotional materials to the FDA at the time of dissemination—even if only minor, non-substantive changes have been made to previously submitted pieces—results in submission of thousands of pieces per company per year with no benefit to public health.

Reforming the 340B Drug Discount Program

To protect the health care safety net it is critical to ensure that the underlying market works. The 340B program needs both Congressional and administrative updates to help prevent it from continuing to raise costs for consumers and the overall health care system. Stronger rules for hospitals participating in the program will help ensure the program targets the patients and true safety-net facilities it was intended to help. Specific reforms for hospitals participating in the program should include stricter 340B eligibility criteria, limits on contract pharmacy arrangements, requirements that patients see a benefit from the program, a tighter definition of patient eligibility, and limits on which hospital-owned physician practices can participate in 340B.

Assuring Robust Competition and Continuing to Modernize the FDA

Economists have reinforced the critical role of boosting competition to address drug cost and access issues. To increase competition:

- Key provisions of the prescription drug, biosimilar, and generic drug user fee acts will spur competition, including policies to eliminate the generic drug application backlog and increased resources to prevent future backlogs, expand FDA's expertise related to drug-device combination products, and reduce the regulatory uncertainty and streamline review of drug-device combination products. Biopharmaceutical companies have stated that current regulatory uncertainties and delays have deterred both generic and brand manufacturers from investments in these areas.

- Reducing the length and increasing the efficiency of drug development will increase competition on both price and clinical effects. Given that the cost of innovator drug development has doubled over the past decade, in part due to increasing FDA requirements, the Prescription Drug User Fee Act includes a range of provisions aimed at reducing uncertainty and creating efficiencies in the both the development and regulatory review of new medicines. Accelerating the introduction of new medicines would allow the forces of private market competition to keep costs in check and increase the number of lifesaving drugs becoming available to patients.
- Enhancing incentives for generic manufacturers to enter the marketplace in areas where there are no intellectual property or regulatory incentives preventing generic entry but due to small population sizes there are no brand or generic competitors. Increased competition from generics could be spurred by waiving user fees for eligible products, providing a transferable generic drug priority review voucher, and expediting review of such products and the inspection of their facilities.
- Finalizing the various FDA guidances related to biosimilars is necessary to reduce regulatory uncertainties for biosimilar manufacturers and to accelerate the market entry of biosimilars. Biosimilar medicines are an important way to spur competition that will lead to more choices for patients and lower prices for patients and the health care system.

Sustaining Incentives for Innovation Is Critical to Solving Future Health Care Challenges

Looking ahead, it is clear that medicines offer some of the clearest opportunities to address the challenge of growing health care costs as our population ages. For example, the number of Alzheimer's cases is projected to increase rapidly over the next decade as Baby Boomers begin to reach retirement 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.⁶¹ 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.⁶² In just the last two years, several promising new therapies failed in mid- and late-stage trials, resulting in the loss of billions of dollars of human, political, and monetary capital.⁶³

⁶¹ 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

⁶² 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>

⁶³ Ogg JC. The List of Failed Alzheimer's Drug Treatments Keeps Growing. 24/7 Wall Street. September 26, 2017. <http://247wallst.com/healthcare-business/2017/09/26/the-list-of-failed-alzheimers-drug-treatments-keeps-growing/>

As with Alzheimer's disease, there is a significant unmet medical need for patients with rare diseases which collectively affect 30 million Americans. But only 5% of these diseases have available treatment options.⁶⁴ Given the many diseases where there is significant unmet need, maintaining incentives for the continued development of new medicines will be crucial in addressing the most costly and challenging diseases of our time.

Yet there is evidence that rising costs in drug development, combined with an increasingly competitive market, have resulted in more uncertainty and lower average returns in recent years. 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.⁶⁵

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.⁶⁶ This underscores the extraordinary risk biopharmaceutical companies confront to bring new treatments to market.

⁶⁴ Pharmaceutical Research and Manufacturers of America (PhRMA). Spurring Innovation in Rare Diseases: 2017 update. http://phrma-docs.phrma.org/files/dmfile/Rare-Disease-Udpate_FINAL.pdf.

⁶⁵ 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.

⁶⁶ Loftus P. Merck Will No Longer Sell its Victrelis Hepatitis C Drug in the US. *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/>