

Sudden Price Spikes in Off-Patent Prescription Drugs: *The Monopoly Business Model that Harms Patients, Taxpayers, and the U.S. Health Care System*



Special Committee on Aging United States Senate

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EXECUTIVE SUMMARY

In May 2015, when Isla Weston was just two months old, doctors diagnosed her with a life-threatening parasitic infection known as toxoplasmosis. Immediate treatment was needed to cure this infection; otherwise, the parasite would attack vital cells in the little girl's brain, potentially leaving her with lifelong deficits in cognition and function—or even causing her death.

Isla was prescribed Daraprim, the standard of care, which would cure the active infection in a year. To the shock and dismay of the infant's family, and other Americans who relied on this vital medicine, the price of the 63-year-old drug that this child desperately needed had just spiked from \$13.50 a tablet to \$750 a tablet, *an increase of more than 5,000 percent*, in just one day.

Testifying at a 2016 Senate Special Committee on Aging hearing, held just a few days after Isla's first birthday, her mother, Shannon Weston, described the impact of that staggering price tag: "I was hopeless and depressed at the thought of what would happen to my perfect little girl if I was not able to help her . . . I looked into any way I could think of to come up with the almost \$360,000 necessary to treat my daughter for a year with a drug that she needed, knowing that as long as she was treated before symptoms set in she would remain asymptomatic."¹

Isla's story is not unique. This family's struggle sadly represents the struggle of thousands of Americans in the face of soaring prescription drug costs. Nearly 60 percent of Americans, including roughly 90 percent of seniors, take prescription drugs to treat conditions ranging from cancer and diabetes to high blood pressure and depression. Staggering increases in the price of some prescription drugs threaten not only the economic stability of American households, but also the health of individuals who discover that drugs they need are unaffordable and difficult to access.

This year alone, Americans are expected to spend more than \$328 billion on prescription drugs. Of this amount, individuals will pay about \$50 billion out of pocket. The federal government will pick up another \$126 billion in payments through Medicare, Medicaid, the Department of Veterans Affairs, and other programs. These price increases affect all Americans, whether they take prescription drugs or not, as taxpayers shoulder a substantial portion of the cost of federal health care programs.

In November 2015, Chairman Susan Collins (R-Maine) and Ranking Member Claire McCaskill (D-Missouri) launched a bipartisan Senate Special Committee on Aging investigation of abrupt and dramatic price increases in prescription drugs whose patents had expired long ago. The Committee's investigation centered on Turing Pharmaceuticals, Retrophin, Inc., Valeant Pharmaceuticals International, Inc., and Rodelis Therapeutics—companies that acquired decades-old, off-patent affordable drugs and then raised the prices suddenly and astronomically.²

¹ *Sudden Price Spikes In Decades Old Rx Drugs: Inside the Monopoly Business Model: Hearing Before the S. Special Comm. on Aging*, 114th Cong., 2d Sess., at 2 (Mar. 17, 2016) (written testimony of Shannon Weston).

² Retrophin post-Mr. Shkreli appears to have repudiated Mr. Shkreli's business model, but has not lowered the price of Thiola.

The investigation uncovered a business model that these four companies used (with some variation) to exploit market failures at the expense of patients. The Committee held three hearings; interviewed scores of patients, doctors, hospital administrators, consumer advocates, health experts, and pharmaceutical industry executives and board members; reviewed more than one million pages of documents obtained from the four companies; and deposed or took transcribed interviews of numerous corporate witnesses.

The first hearing of the series, held on December 9, 2015, sought to identify and define the problems resulting from these price increases. The second hearing, held on March 17, 2016, at which Shannon Weston testified, took an in-depth look inside the monopoly business models of Turing and Retrophin, both formerly headed by Martin Shkreli, who was dubbed “pharma-bro” by the media. The third hearing, held on April 27, 2016, investigated Valeant’s business model, its investor relationships, and the harm caused to patients and the health care system by the enormous price increases Valeant imposed on certain drugs it acquired.

This Report closely examines the business model used by these companies; provides case studies of the four companies; explores the influence of investors; assesses the impacts of price hikes on patients, payers, providers, hospitals, and governments; and discusses potential policy responses.

The Business Model

The Committee discovered that each of the four companies followed a business model (with some variation) that enabled them to identify and acquire off-patent sole-source drugs over which they could exercise de facto monopoly pricing power, and then impose and protect astronomical price increases. The business model consists of five central elements:

Sole-Source. The company acquired a *sole-source drug*, for which there was only one manufacturer, and therefore faces no immediate competition, maintaining monopoly power over its pricing.

Gold Standard. The company ensured the drug was considered the *gold standard*—the best drug available for the condition it treats, ensuring that physicians would continue to prescribe the drug, even if the price increased.

Small Market. The company selected a drug that served a *small market*, which were not attractive to competitors and which had dependent patient populations that were too small to organize effective opposition, giving the companies more latitude on pricing.

Closed Distribution. The company controlled access to the drug through a *closed distribution system* or specialty pharmacy where a drug could not be obtained through normal channels, or the company used another means to make it difficult for competitors to enter the market.

Price Gouging. Lastly, the company engaged in *price gouging*, maximizing profits by jacking up prices as high as possible. All of the drugs investigated had been off-patent for decades, and none of the four companies had invested a penny in research and development to create or to significantly improve the drugs. Further, the Committee found that the companies faced no meaningful increases in production or distribution costs.

Case Studies of the Four Companies

Table 1 provides an overview of case studies from Turing, Retrophin, Rodelis, and Valeant. Each company selected a sole-sourced gold standard drug for which there is a small market, created a closed distribution system or other means to block competitors, and engaged in price gouging, exercising elements of the business model to make massive profits from decades-old life-saving therapies.

Turing raised the price of Daraprim, the gold standard for toxoplasmosis, from \$13.50 a pill to \$750 a pill, and put the drug in a closed distribution system to keep potential generic competitors from getting access to the drug to conduct required bioequivalence tests for developing generic alternatives. Retrophin raised the price of Thiola, the preferred therapy for cystinuria, a rare, chronic, genetic kidney disease, from \$1.50 a tablet to \$30 per tablet, and also instituted a closed distribution system. Rodelis raised the price of Seromycin, the gold standard for multi-drug resistant tuberculosis, from \$500 for 30 capsules to \$10,800 for 30 capsules.

Valeant, the largest of the companies investigated, presents the most complex case. This company spiked the price of not one off-patent gold standard drug, but four. Valeant raised the prices of Cuprimine and Syprine, two drugs that treat Wilson disease, the rare genetic inability to process copper, from about \$500 to about \$24,000³ for a 30-day supply. Both drugs became supported via an exclusive patient assistance program designed to attract and retain high-value patients (cash paying or private insurance). This program arguably left potential competitors with little prospect of making a profit if they entered. Valeant also raised the price of Nitropress and Isuprel, two hospital drugs that are life-saving in emergency cardiac cases, from approximately \$2,000 to \$8,800 and \$17,900, respectively.⁴

³ These figures are approximate. The exact prices of Cuprimine and Syprine are listed in Table 1.

⁴ These figures are approximate. The exact prices of Nitropress and Isuprel are listed in Table 1.

Table 1. Case Studies of the Four Companies

	Sole-Source	Gold Standard	Small Market	Closed Distribution	Price Gouge
Turing Pyrimethamine, the Active Pharmaceutical Ingredient (API) for Daraprim was developed in 1953.	Daraprim was a sole-source drug, and Turing attempted to lock up the supply of its API, pyrimethamine, to ensure it remained so.	Daraprim is the “gold standard” for treating toxoplasmosis.	Has small patient population (congenital or immune-suppressed adults).	Implemented closed distribution to keep generic companies from obtaining Daraprim necessary to develop generics.	Increased the price of Daraprim from \$13.50 to \$750 per pill— increase of more than 5,000%.
Retrophin Thiola went to market in 1988.	No known generic competitors to Thiola at time of acquisition.	Thiola was one of two drugs approved for cystinuria, but is considered the “preferred therapy.”	Cystinuria is a rare disease. Retrophin estimated only 300 to 400 patients were on Thiola.	Retrophin said “[c]losed distribution system prevents generics from accessing the product for bioequivalence studies.”	Increased the price of Thiola from \$1.50 to \$30 per tablet—an increase of nearly 2,000%.
Rodelis <i>(acquisition was reversed weeks after the price increase)</i> Seromycin was brought to market in 1964.	There were no generic competitors to Seromycin.	Treats multi-drug resistant tuberculosis—“the only drug approved for MDR that treats both pulmonary and extra-pulmonary TB.”	A very small number of cases of MDR TB per year in the U.S.—most experts estimate in the hundreds.	Intended to pursue “[s]everal defensive mechanisms and barriers to entry for generic competition.”	Increased the price of Seromycin from \$500 to \$10,800 for 30 capsules— 2,060% increase.
Valeant Cuprimine—1956, Food and Drug Administration (“FDA”) approved in 1965. Syprine—1969, FDA approved in 1985.	Syprine is the only trientine hydrochloride available for the treatment of Wilson disease.	Experts consider Syprine (and to a lesser extent, Cuprimine) to be the gold standard for treating Wilson disease.	Wilson disease is very rare—about 2,000—3,000 cases in the U.S.	Established patient assistance program to attract and retain high-value patients (cash paying / private insurance).	Increased price of Cuprimine from \$445 to \$26,189—5,785% increase; Syprine from \$652 to \$21,267—3,162% increase.
Valeant Isuprel—patented 1956. Nitropress—active ingredient isolated in 19th C.	Valeant viewed the drugs as effectively sole-source, <i>i.e.</i> , “the only options available,” and accordingly believed they had more pricing power.	Both drugs: “[c]onsidered standard of care,” and “must be available in limited situations where needed.”	Both are used in hospitals in emergency settings.	Valeant expected generics to enter, but calculated FDA processing delays would create de facto monopoly for years.	Increased the price of Isuprel from \$2,183.00 to \$17,901.12 for ten 5 mL vials— 720% increase; Nitropress from \$2,148.30 to \$8,808.80 for ten 2 mL vials— 310% increase.

Role of Investors

The business model employed by the four companies appears in some instances to have been actively supported and promoted by investors. Additionally, many of the companies were headed by senior management lacking in a pharmaceutical background and hailing from the hedge fund world. This may help explain why these companies may have been run more like hedge funds than pharmaceutical companies.

In the case of Retrophin, internal emails reveal how Dan Wichman, an investor from Broadfin Capital, outlined the business model to Mr. Shkreli, who at the time was Chief Executive Officer (“CEO”) of Retrophin: “Funny that these small companies still haven’t realized you can raise price aggressively and nobody gets too upset?”⁵

Mr. Shkreli’s next hedge fund pharmaceutical venture, Turing, was notable for being run by those who lacked pharmaceutical experience, but had ample experience investing and running hedge funds. Mr. Shkreli hailed from the world of hedge funds and lacked any pharmaceutical experience. So too did his handpicked successor, Ron Tilles, a broker by training whose main skillset was soliciting investors, and who, by his own admission, did not know the most basic of pharmaceutical concepts when deposed a week before the Committee’s March 2016 Hearing. In Rodelis, the boundaries between the company and its largest investor, Avego Healthcare Capital, were practically invisible—individuals holding senior offices in both companies took on interchangeable roles. These individuals actively drove Rodelis’ efforts to use the business model to create profits.

Patient and Family Impacts

Sudden price spikes in decades-old drugs have devastated patients and families across the nation. Dozens of Americans called the Committee to share their stories. Patients have been forced to go without vital medicine, resulting in potentially mortal peril. Patients reported having to skip doses or hoard pills. Poignantly, patients reported the anxiety they felt as they watched prices climb, knowing that they could lose access without warning. The drug could get dropped from an insurance plan formulary; an application for a patient assistance program (“PAP”) could get denied; a foundation grant could run dry—everyday, in the face of these price spikes, patients and their families live in fear of future untold shock.

When the patients themselves are too ill to clear the hurdles imposed by PAPs, family members champion their struggles. Several individuals likened the paperwork requirements for PAPs, which require continually reapplying and following up, to having a part-time job. Several also took on second jobs to help cover the increased cost of treatment, which often persists even when help arrives through PAPs or insurance coverage.

The Committee heard from Americans of all ages and all backgrounds, from young couples with infants struggling to make ends meet to grandparents with retirement on the horizon. Berna Heyman, a retiree who testified before the Committee, had been living with Wilson disease that she controlled with Syprine three times a day. One day in 2014, she realized

⁵ Email from Dan Wichman to Martin Shkreli, SSICA_THIOL_037898, at SSICA_THIOL_037903 (Mar. 6, 2014).

that her projected co-pay would exceed \$10,000 per year—with her insurance paying more than \$260,000—and that such costs were untenable for her, despite having a good insurance plan. After a series of attempts to obtain patient assistance to no avail, Mrs. Heyman ultimately took a risk and switched to a zinc-based drug. She testified, “[m]y health was stable with Syprine and my doctor and I made the change only under duress.”⁶

Hospital and Community Impacts

Hospitals interviewed by the Committee have also been forced to make extensive changes, while simultaneously facing significant uncertainties and suffering enormous budget repercussions from the price spikes. Nitropress and Isuprel, two Valeant drugs, contributed greatly to the overall rising cost of drugs for hospitals. The Ascension Health System, for example, reported a \$12 million budgetary impact in 2015 from pharmaceutical price increases, with Nitropress and Isuprel ranking first and second among the hospital drugs that were contributing to its increased costs. The Johns Hopkins Health System reported it suffered a \$1 million hit in 2015 from price increases for Nitropress and Isuprel, and the Cleveland Clinic spent over \$5 million for the two drugs in 2015, compared to less than \$2 million the prior year.

In an effort to reduce costs, these hospitals have taken aggressive steps to reduce their usage of Nitropress and Isuprel: cutting back or eliminating the use of Isuprel on hospital emergency “crash carts”; substituting other drugs where possible; actively seeking alternative approaches; aggressively monitoring usage; and reducing inventories. Achieving these reductions is itself a costly process. The hospital representatives reported that making the change is not as simple as substituting a new drug in the pharmacy. Administrators had to develop new policies and protocols as well as train medical professionals in the proper use of the drug, many of whom had been using Nitropress and Isuprel for decades. The increased time that administrators, physicians, nurses, and others who treat patients spend developing policies and learning and implementing new protocols is time away from patient care.

Dr. Richard Fogel, Chief Clinical Officer of St. Vincent’s Hospital in Indiana, testified at the Committee’s April 2016 Hearing that increased hospital spending on Nitropress and Isuprel would cause the institution to cut back on providing health care services to the broader community. Dr. Fogel cited expansion of the hospital’s Rural and Urban Access to Health initiative, which connects low-income and vulnerable communities with health care services, food, transportation, and housing, as well as a number of initiatives to fight the opioid epidemic, as casualties of this price increase. The price spikes harm not only patients at the hospital, but also the entire community around the hospital. Dr. Fogel testified, “We have seen more of these hospitals close because the financing was simply unsustainable.” Hospital closings cause untold hardships for the communities they serve.

⁶ *Valeant Pharmaceutical’s Business Model: The Repercussions for Patients and the Health Care System: Hearing Before the S. Special Comm. on Aging*, 114th Cong., 2d Sess., at 2 (Apr. 27, 2016) (written testimony of Berna Heyman).

Policy Responses

The Committee's investigation focused on four pharmaceutical companies, and evidence gathered by the Committee suggests that additional companies have employed the business model uncovered in this report, forcing Americans to make difficult decisions about their health due to financial constraints. This troubling practice must be stopped to help rein in price spikes in off-patent, decades-old drugs purchased by companies that did not bear the research and development costs for these drugs. The Committee evaluated a number of potential policy solutions and considered the views of a wide-range of health policy experts and clinicians. With an issue as complex as drug pricing, members understandably have differing views on the merits of the various options available to policymakers, including the responses described in this report. While release of the report does not indicate unanimous support of each of these policy options, we hope that it will help contribute to the ongoing discussion:

Enact the Increasing Competition in Pharmaceuticals Act of 2016 to provide solutions to regulatory uncertainty, small market size, and other factors that serve as limitations to generic entry by incentivizing competition. Introduced by Chairman Collins and Ranking Member McCaskill, this bipartisan bill sets a clear timeframe of 150 days for the Food and Drug Administration (“FDA”) to expedite review of certain generic applications and provides an incentive in particular cases in order to keep the marketplace competitive, drug prices down, and improve access for patients. The introduction of the bill has already resulted in a success—after its introduction, the FDA announced that it would prioritize administratively the review of generic applications for certain off-patent prescription drugs for which there is only one manufacturer, a key provision of the bill.

Encourage generic competition by ensuring the right to obtain samples and simplifying Risk Evaluation and Mitigation Strategies (“REMS”). The *Creating and Restoring Equal Access to Equivalent Samples Act of 2016*, sponsored by Senate Judiciary Committee Ranking Member Patrick Leahy (D-Vermont) and Chairman Chuck Grassley (R-Iowa), and cosponsored by Senators Collins and McCaskill, would provide a mechanism by which a potential generic entrant could commence expedited litigation to obtain access to samples of the reference listed drug required for FDA approval for a potential generic competitor. Additionally, the Committee believes that the FDA should be allowed to exercise its discretion to allow potential generic entrants to create their own REMS system, instead of relying on the current single shared REMS system of the reference listed drug.

Allow highly targeted temporary drug importation to combat major price increases in off-patent drugs to provide prompt price relief. The Committee believes that temporary importation may be a viable short-term solution to combat sudden price spikes, but notes that while this approach is favored by a number of academic professionals, many caution that care must be taken in structuring such a regime to avoid unintended consequences.

Prevent the misuse of patient assistance programs and copay coupons. The Committee found that self-serving motives were often critical to understanding patient assistance programs and is concerned that patient assistance can be used to steer patients toward higher priced drugs, resulting in higher expenditures for beneficiaries, federal health care programs, and commercial providers. The Committee finds this issue warrants further study.

Reinvigorate the Federal Trade Commission (“FTC”) to enforce action when it comes to drug company mergers, operations, and drug market dynamics. The Committee encourages the FTC to explore greater use of its existing authority and to conduct studies of the marketplace; to consider partnerships with academia and other federal agencies; and to work with the U.S. Department of Health and Human Services, the Department of Justice, and the FDA to promote complementary work and harmonization between agencies. Based on the Committee’s review, the FTC needs more resources to allow it to more vigorously oversee the off-patent prescription drug market.

Improve transparency in the health care system. The lack of transparency in drug prices is omnipresent in the prescription drug industry. Releasing, for example, the true price of a drug, the Average Manufacturer Price, after a lag period could empower patients and doctors, prevent surprise costs at the pharmacy or on health bills, and provide Americans with a refreshing dose of reality.

Conclusion

For many decades, federal policy has sought to strike the right balance between maintaining the incentives needed to promote innovation and development of new drugs, and keeping medicines affordable for patients. This balance has been struck by allowing a period of patent protection for innovative drugs, and then opening the market to generic competition to help drive down prices. On average, generics cost 80 percent less than brand-name drugs. That balance never anticipated companies acquiring off-patent drugs, for which they contributed not a single research and development dollar, and then dramatically increasing their prices in the absence of generic competitors. This investigation has shed light on why such companies can impose egregious price increases on off-patent drugs they have acquired and what federal policies should be considered to counter this disturbing practice.

This investigation has brought to light the stories of infants like Isla and seniors like Mrs. Heyman. Isla ultimately received treatment, not through affordable access to Daraprim, but through the genius and goodwill of her health care team at the University of North Carolina. Mrs. Heyman ultimately found affordable treatment by switching to an alternate therapy, but in doing so, she endures lifestyle restrictions and uncertainties about future effectiveness of the drug. Americans are continuing to struggle with high drug costs.

During the course of the Committee's investigation, other companies raised their prices sharply. In April 2016, a study found that the mean price of insulin, a lifeline therapy for the 29 million Americans with diabetes, increased from \$4.34 per milliliter in 2002 to \$12.92 per milliliter in 2013, a 200 percent increase. In July 2016, a flurry of news stories reported another staggering price spike: the price of Naloxone, the antidote to prescription painkiller overdoses, increased by 1,000 percent, amid an opioid public health crisis. And in August 2016, news broke of a 500 percent price spike in the epinephrine auto-injector, EpiPen, which is used to save lives during allergy emergencies.

The cost of prescription medications continues to be of great concern to the American public. For every baby born tomorrow and every American who reaches retirement today, the Senate Special Committee on Aging is committed to improving access and affordability of prescription medications. The Committee strongly supports continued efforts to stop the bad actors who are acquiring drugs that have been off-patent for decades, and then driving up their prices, to paraphrase Mr. Shkreli, "because I can."

CHAPTER 1. BACKGROUND

I. Purpose and Scope of the Investigation

Nearly 60 percent of Americans take prescription drugs to treat conditions ranging from cancer and diabetes to high blood pressure and depression.⁷ These medications are vital to the health and well-being of Americans. This is especially true of our nation's seniors, approximately 90 percent of whom take at least one prescription drug in any given month.⁸ Soaring increases in the price of some prescription drugs threaten not only the economic stability of American households, but also the health of individuals who find that vital drugs are unaffordable and difficult for them to access. This year alone, Americans are expected to spend more than \$328 billion on prescription drugs.⁹ Of this amount, individuals will pay more than \$45 billion out-of-pocket.¹⁰ The federal government will pick up another \$126 billion in payments through Medicare, Medicaid, Department of Veterans Affairs, and other programs.¹¹ These price increases affect Americans, whether they take prescription drugs or not, as taxpayers shoulder a substantial portion of the cost of federal health care programs.

In November 2015, Chairman Susan Collins (R-Maine) and Ranking Member Claire McCaskill (D-Missouri), launched a bipartisan Senate Special Committee on Aging investigation focused on abrupt and dramatic price increases in decades-old prescription drugs that are no longer protected by patents or other legal exclusivity. In particular, the Committee's investigation centered on pharmaceutical companies that devised their business models to acquire a drug over which they could exercise de facto monopoly pricing power due to a market failure, and then impose and maintain astronomical price increases. The Committee also explored potential policy changes to respond to these market failures. The Committee held three hearings; interviewed patients, doctors, hospital administrators, consumer advocates, health experts, pharmaceutical industry executives and board members; reviewed more than one million pages of documents obtained from four companies—Turing Pharmaceuticals LLC (“Turing”), Retrophin, Inc. (“Retrophin”), Valeant Pharmaceuticals International, Inc. (“Valeant”), and Rodelis Therapeutics (“Rodelis”); and deposed or took transcribed interviews of ten corporate witnesses.

⁷ See Elizabeth M. Kantor, et al., *Trends in Prescription Drug Use Among Adults in the United States from 1999–2012*, 314 J. Am. Medical Assoc., 1818, 1818 (Nov. 3, 2015).

⁸ Linda Barret, *Prescription Drug Use Among Midlife and Older Americans* (Jan. 2005); Dima M. Qato, et al., *Changes in Prescription and Over-the-Counter Medication and Dietary Supplement Use Among Older Adults in the United States, 2005 vs 2011*, 176 J. Am. Medical Assoc. 473, 473 (Apr. 2016).

⁹ See U.S. Dep't of Health & Human Servs., Office of the Assistant Secretary for Planning and Evaluation, *Observations on Trends in Prescription Drug Spending*, at 1 (Mar. 8, 2016), *found at*, <https://aspe.hhs.gov/sites/default/files/pdf/187586/Drugspending.pdf> (last visited Dec. 9, 2016).

¹⁰ See U.S. Dep't of Health & Human Servs., Ctr. for Medicare & Medicaid Servs., *Prescription Drug Expenditures*, National Health Expenditures by Type of Service and Source of Funds, CY 1960-2015, line 284, *found at*, <https://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/NationalHealthExpendData/NationalHealthAccountsHistorical.html> (last visited Dec. 12, 2016).

¹¹ *Id.* at lines 287, 289, 292, 294, 295, 299, 302, and 308, which totals to \$126.246 billion.

The first hearing of the series, held on December 9, 2015, sought to identify and define the problems resulting from these price increases.¹² The second hearing, held on March 17, 2016, took an in-depth look inside the monopoly business models of Turing and Retrophin, both formerly headed by executive Martin Shkreli.¹³ The third hearing, held on April 27, 2016, investigated Valeant's business model, its investor relationships, and the harm caused to patients and the health care system by the enormous price increases Valeant imposed on certain drugs it acquired.¹⁴

For many decades, federal policy has sought to strike the right balance between maintaining the incentives needed to promote the innovation and development of new drugs, and keeping medicines affordable for patients. That balance did not account for companies acquiring off-patent drugs, for which they played no role in the research and development, and then dramatically increasing their prices in the absence of generic competitors. A chief goal of the Committee's investigation has been to understand why such companies can impose egregious price increases on off-patent drugs they have acquired and what federal policies should be considered to counter this disturbing practice.

This Report closely examines the business model used by these companies; summarizes case studies from the four companies; assesses the impacts of price hikes on patients, payers, providers, hospitals, and governments; and discusses potential policy responses.

II. An Overview: Drug Pricing in the United States

Pharmaceutical companies take into account a number of factors when deciding what price to set for their drugs. These factors include the market for a particular type of drug, the cost of comparative treatments for a disease, the cost of supporting current and future research and development, the price of manufacturing and ingredients, and how to maximize profits. The market for a particular drug plays a crucial part in benchmarking where its price will be set. A manufacturer generally will not set the price beyond what the market will bear for its product at the risk of losing market share. This is particularly true in the case of off-patent drugs for which there are a number of competitors in the market. Regardless of how much a manufacturer may want to maximize profits in that instance, market competition will likely keep the price low.

Although many drugs have a well-defined market and clear competitors (*e.g.* statins, anti-histamines, pain killers, etc.), there are many other drugs that do not because they are the only drugs of their kind. These drugs run the gamut from the truly innovative drugs that are the first to cure a disease (*e.g.*, Sovaldi, which is used to treat hepatitis C virus infection) to older

¹² *Sudden Price Spikes in Off-Patent Drugs: Perspective from the Front Lines: Hearing Before the S. Special Comm. on Aging*, 114th Cong., 1st Sess. (Dec. 9, 2015) (hereinafter "December 2015 Hearing").

¹³ *Sudden Price Spikes In Decades Old Rx Drugs: Inside the Monopoly Business Model: Hearing Before the S. Special Comm. on Aging*, 114th Cong., 2d Sess. (Mar. 17, 2016) (hereinafter "March 2016 Hearing"). As discussed, *infra*, at 41–42, Retrophin post-Mr. Shkreli appears to have repudiated Mr. Shkreli's business model, but has not lowered the price of Thiola.

¹⁴ *Valeant Pharmaceutical's Business Model: The Repercussions for Patients and the Health Care System: Hearing Before the S. Special Comm. on Aging*, 114th Cong., 2d Sess. (Apr. 27, 2016) (hereinafter "April 2016 Hearing").

drugs that have been around for decades without any generic competition. For the innovative drugs, patent protection and the U.S. Food and Drug Administration (“FDA”) market exclusivity provide a timeframe within which a manufacturer can charge whatever it wishes for a drug. These innovative drugs rely mostly on their therapeutic value to gain market share once they enter the market. On the other hand an off-patent drug generally has no monopoly protection, and in the event it has generic drug competition, it is supposed to compete for market share based on price.¹⁵

There are a large number of drugs, however, that are off-patent and yet face no generic competition. In the case of these sole-source drugs, the manufacturer enjoys a de facto monopoly and there is no market force to prevent the manufacturer from charging whatever it wishes for the drug.

The prescription drug industry consists of an opaque and complex network of entities engaged in multiple distribution and payment structures for drugs. These entities include pharmaceutical companies, wholesalers, pharmacies, third-party payers that provide insurance coverage, pharmacy benefit managers, and consumers. Additionally, group purchasing organizations negotiate contracts with vendors on behalf of a large number of hospitals or other providers.

Pharmaceutical Companies. Pharmaceutical companies that own the rights to manufacture and market drugs are also known as “drug manufacturers,” even if they contract out the actual production of the prescription drugs.¹⁶ Rights can be original to the company that invested in the research and development of the drug, or they can be acquired at any stage during the development of the drug or after it has come on the market.¹⁷ Pharmaceutical companies typically own or contract with facilities that manufacture the drugs, and then sell their product to wholesalers.¹⁸

Wholesalers. After production, many manufacturers send their drugs to FDA-registered drug wholesalers for further distribution.¹⁹ Wholesalers act as distributors: purchasing, inventorying, and selling pharmaceutical products to a variety of providers, including retail pharmacy outlets, hospitals, and clinics.²⁰ States license or authorize wholesalers that sell and

¹⁵ In this report, an “off-patent drug” is a drug that is not currently under patent protection and a “generic drug” is one that is a biological equivalent to another drug (it is worth noting that as generic drugs do not have patent protection, they are also technically off-patent drugs). A manufacturer can make a generic drug copy of an off-patent drug, but not all off-patent drugs have a generic drug that is its copy.

¹⁶ See U.S. Dep’t of Commerce Int’l Trade Admin., *Pharmaceutical Industry Profile*, at 1 (July 2010), found at, <http://www.trade.gov/td/health/PharmaceuticalIndustryProfile2010.pdf> (last visited Dec. 9, 2016).

¹⁷ See Mark Kessel, *The Problems with Today’s Pharmaceutical Business—an Outsider’s Perspective*, 29 *Nature*, 27, 27, (Jan. 2011).

¹⁸ See, e.g., Ernst Berndt and Joseph Newhouse, *Pricing and Reimbursement in U.S. Pharmaceutical Markets*, Harvard Kennedy School, National Bureau of Economic Research, 8, (Sept. 2010).

¹⁹ *Id.*

²⁰ See FDA, *Guidance for Industry: Prescription Drug Marketing Act Requirements*, at 3 (Nov. 2006), found at, <http://www.fda.gov/downloads/drugs/guidancecomplianceregulatoryinformation/guidances/ucm134399.pdf> (last visited Dec. 11, 2016).

distribute pharmaceuticals within their borders.²¹ The wholesaler market in the United States is dominated by three companies: AmerisourceBergen Corp., Cardinal Health Inc., and McKesson Corp.²²

Third-Party Payers. Third-party payers submit payments on behalf of insured individuals to health care providers for services rendered.²³ Third-party payers include self-insured businesses; insurance companies, such as insurers that participate in Medicaid and Medicare; and union-run health plans.²⁴ These health care payers span public and private insurance programs as well as managed care and preferred provider networks.²⁵

Pharmacy Benefit Managers. Pharmacy benefit managers (“PBMs”) act as intermediaries between manufacturers and health care payers.²⁶ PBMs handle a variety of services, including prescription billing, the negotiation of drug prices with drug companies, and the creation of retail pharmacy networks for insurers, including contracting with mail-order pharmacies and negotiating reimbursement rates with them.²⁷

PBMs also design their own formularies, which are lists of drugs covered by a PBM and its members.²⁸ In determining which drugs to cover, a PBM groups drugs it considers to be therapeutically similar.²⁹ For drugs with several close substitutes, a PBM negotiates with manufacturers for rebates in return for placing the manufacturers’ drugs on their formularies or giving the drugs preferential placement.³⁰ Preferential placement may entail charging a lower co-payment for the preferred drugs compared to other (non-preferred) drugs that are therapeutically similar.³¹ The PBMs can have a significant impact on the price of a drug, as well as the drug market as a whole, as they essentially control access to a drug for large portions of the health care market through their formularies and negotiated contracts. PBMs generate income through service fees from large customer contracts for processing prescriptions, operating mail-order pharmacies, and from spreads off of rebates negotiated with drug makers. Their contracts can include incentives for cutting costs.³² The largest PBMs include Express Scripts, CVS Health Corp, UnitedHealth Group, and Catamaran.³³

²¹ See FDA, Profile of the Prescription Drug Wholesaling Industry, at 3 (undated), *found at*, <http://www.fda.gov/ohrms/dockets/dockets/05n0403/05n-0403-bkg0001-04-02-1.pdf> (last visited Dec. 11, 2016).

²² See Susan Thaul, Cong. Research Serv., R43106, *Pharmaceutical Supply Chain Security*, at 4 (Oct. 31, 2013).

²³ See Thomas Bodenheimer, *High and Rising Health Care Costs. Part 1: Seeking an Explanation*, 142 Ann Intern Med. 847, 847 (May 17, 2005).

²⁴ *Id.*

²⁵ See Sherry Glied, *Chapter 13—Managed Care*, in *Handbook of Health Economics*, 708, 709 (Anthony J. Culver and Joseph P. Newhouse 1A. 2000).

²⁶ See Gryta Thomas, *What is a “Pharmacy Benefit Manager”*, The Wall St. J. (July 21, 2016).

²⁷ *Id.*

²⁸ See Robert F. Atlas, *The Role of PBMs in Implementing the Medicare Prescription Drug Benefit*, 23 Health Affairs, w4-504, w4-507 (July 2004).

²⁹ *Id.*

³⁰ *Id.*

³¹ *Id.*

³² *Id.*

³³ See *Pharmacy-Benefit Managers*, The Wall St. J. (Mar. 30, 2015) *found at*, <http://blogs.wsj.com/briefly/2015/03/30/pharmacy-benefit-managers-the-short-answer/> (last visited Dec. 15, 2016).

Group Purchasing Organizations. Group purchasing organizations (“GPOs”) negotiate contracts with wholesalers or manufacturers on behalf of a large number of hospitals or other providers.³⁴ GPOs may be used by hospitals and other providers to purchase a range of goods and services, including drugs, hospital equipment, and high technology products.³⁵ As a result of their large member base, GPOs are able to negotiate much more favorable prices with wholesalers/manufacturers for a particular item or service than an individual hospital or provider could on its own.³⁶ A GPO’s main source of operating income comes from “contract administrative fees,” a fixed percentage paid by the supplier to the GPO as part of closing a specific sale between supplier and hospital.³⁷ These contract administrative fees are typically a percentage of the costs of the products that a GPO is purchasing from a wholesaler for its members through a GPO-negotiated contract.³⁸ Ninety percent of hospitals use national GPOs.³⁹ Many hospitals also use regional and local GPOs in addition to national GPOs.⁴⁰ Even if a hospital is a member of a GPO, it will typically self-negotiate for some products.⁴¹

Figure 1, which appears at the end of this section, illustrates some of the different entities and the common relationships among them. While payment varies from drug to drug, the basic payment structure follows this pattern: The patient pays the health insurer (via their health insurance premium), which pays the pharmacy, which pays the pharmaceutical drug company. Similarly, while distribution systems vary across drugs, the common structure involves dispensing drugs to patients via retail and mail order pharmacies as the figure illustrates.⁴²

Payment Structure. When a drug is dispensed to a patient, the insurer or health plan pays the pharmacy. The pharmacy obtains the drugs from wholesalers, which have purchased them directly from the manufacturers, the pharmaceutical companies.⁴³ Many of these transactions are opaque because the cost from one party to the next is not made known and there are overlapping factors that influence price.⁴⁴

³⁴ See Carl A. Johnston & Curtis D. Rooney, *GPOS and the Health Care Supply Chain: Market-Based Solutions and Real-World Recommendations to Reduce Pricing Secrecy and Benefit Health Care Providers*, 29 J. Contemp. Health L. & Pol’y 72, 75 (2012).

³⁵ *Id.* at 80.

³⁶ *Id.* at 79.

³⁷ *Id.* at 81.

³⁸ GPOs are allowed to collect such contract administrative fees as long as they meet the requirements of a safe harbor to the Anti-Kickback Act (“AKA”). See 42 U.S.C. § 1320a-7b(b) (2006). The AKA would otherwise prohibit such fees.

³⁹ See Lawton Robert Burns and Rada Yovovich, *Hospital Supply Chain Executives’ Perspectives on Group Purchasing: Results from a 2014 Survey*, at 6 (Sept. 2014).

⁴⁰ *Id.*

⁴¹ Committee Staff Interview with Erin Fox, Pharm. D. (University of Utah) (Nov. 5, 2015) (“Fox Interview”).

⁴² U.S. Gov’t Accountability Office, *Generic Drugs Under Medicare*, GAO-16-706, at 7 (Aug. 2016).

⁴³ See U.S. Dep’t of Health & Human Servs., *The Assistant Secretary for Planning and Evaluation, Prescription Drug Prices*, at 100 (Apr. 1, 2000), found at, <https://aspe.hhs.gov/sites/default/files/pdf/172171/c3.pdf> (last visited Dec. 11, 2016).

⁴⁴ See Uwe E. Reinhardt, *The Pricing of U.S. Hospital Services: Chaos Behind a Veil of Secrecy*, 25 Health Affairs 57, 58 (Jan. 2006).

When a manufacturer sets a price for its product, it is generally known as the wholesale acquisition cost (“WAC”).⁴⁵ This is the manufacturer’s list price for sale to wholesalers.⁴⁶ Depending on the volume being sold to the wholesaler, the manufacturer may provide some rebates or discounts to the wholesaler.⁴⁷ The wholesaler handles the sale and distribution of drugs to both retail and non-retail (hospitals, clinics, etc.) pharmacies.⁴⁸ Pharmacies may negotiate rebates or discounts with wholesalers or manufacturers if a manufacturer is selling directly to the pharmacy.⁴⁹ In order to maximize their purchasing power, non-retail pharmacies belonging to hospitals, nursing homes, and other health care systems will typically use GPOs to negotiate further discounts with the wholesaler.⁵⁰ This complex price system can lead to different entities paying different prices for the same drug.⁵¹

The discrepancy between the marketed price and the actual price of drugs is further obscured by confidential agreements between the drug company and the purchaser, which may include chargebacks, rebates, stocking allowances, and a number of other discounts.⁵² Since these agreements are confidential, the various parties involved in these transactions typically do not know what other parties paid or earned for their role in the flow of money.⁵³

From the lens of the individual consumer, the health payment system relies largely on cost-sharing.⁵⁴ Most consumers purchase insurance coverage from a third-party payer, including private health insurance plans, such as those offered by employers, or public plans, such as those offered by the federal government.⁵⁵ The consumer typically pays a fixed monthly amount to the health insurance plan, plus a co-payment for medical visits or medications, tiered based on an established contractual agreement.⁵⁶ The plan sponsors determine formulary coverage, copayment tiers utilization management, and pharmacy channel options.⁵⁷ Because most people

⁴⁵ See Medicaid and CHIP Payment Access Comm., *Medicaid Payment for Outpatient Prescription Drugs*, Issue Brief, at 2 (Sept. 2015).

⁴⁶ *Id.*

⁴⁷ See Richard G. Frank, *Prescription Drug Prices: Why Do Some Pay More Than Others Do?* 20 *Health Affairs*, 115, 124 (Mar. 2001).

⁴⁸ *Id.*

⁴⁹ *Id.* at 121.

⁵⁰ See Carl A. Johnston & Curtis D. Rooney, *GPOS and the Health Care Supply Chain: Market-Based Solutions and Real-World Recommendations to Reduce Pricing Secrecy and Benefit Health Care Providers*, 29 *J. Contemp. Health L. & Pol’y* 72, 79 (2012).

⁵¹ See Richard G. Frank, *Prescription Drug Prices: Why Do Some Pay More Than Others Do?* 20 *Health Affairs*, 115, 115 (Mar. 2001).

⁵² U.S. Dep’t of Health and Human Servs., The Assistant Secretary for Planning and Evaluation, *Prescription Drug Prices*, at 95 (Apr. 1, 2000), *found at*, <https://aspe.hhs.gov/sites/default/files/pdf/172171/c3.pdf> (last visited Dec. 11, 2016).

⁵³ *Id.*

⁵⁴ See Robert P. Navarro and Rusty Hailey, *Overview of Prescription Drug Benefits in Managed Care*, at 17, in, Robert Navarro, *Managed Care Pharmacy Practice* (2nd Ed. 2009).

⁵⁵ *Id.*

⁵⁶ See AHIP Foundation, *A Consumer’s Guide to Understanding Health Plan Networks*, at 20, *found at*, <http://www.ahipfoundation.org/Interactive-Consumer-Guide.pdf> (last visited Dec. 10, 2016).

⁵⁷ See Anna Cook, Julie Somers, and Julia Christensen, *Prescription Drug Pricing*, Congressional Budget Office, at 5, (Jan. 30, 2009), *found at*, http://www.nhpf.org/library/handouts/Cook.slides_01-30-09.pdf (last visited Dec. 11, 2016).

have health insurance, they are direct payers only for the deductible and co-pay portions of their health care expenses.⁵⁸ When a patient either picks up their prescription from a retail pharmacy or receives a prescription drug from a hospital procedure, the patient will typically pay a co-pay to the pharmacy, and the pharmacy receives the balance of the cost from the patient's insurance company (the payer).⁵⁹ This, however, doesn't reflect the true payment that the insurer has made on behalf of the patient, as the insurer has negotiated prices for these drugs and receives additional rebates from its PBM.⁶⁰ The PBMs provide the rebates to insurers because they have in turn negotiated prices and rebates from manufacturers on behalf of insurers (while taking a cut of the rebates).⁶¹

For drugs that are not covered by an individual's health insurance plan, the patient may seek alternate sources of financial support, including from patient assistance programs and patient access network grants.⁶² Patient assistance programs are often funded and run by pharmaceutical companies.⁶³ Patient assistance network grants are often run by independent non-profit foundations, which may receive financial support from pharmaceutical companies.⁶⁴ Both provide support directly to individuals.⁶⁵ These programs typically include eligibility and authorization criteria that are renewed on a monthly or annual basis, and specific parameters vary depending on the program and the drug.⁶⁶ Most programs include criteria regarding income to better serve lower-income individuals.⁶⁷ All of these programs by statute exclude individuals who are on federally funded health programs, such as Medicare or Medicaid.⁶⁸ Patient assistance programs serve as a way to bypass insurance or obtain drugs at a lower cost when insurance coverage is inadequate.⁶⁹ Additionally, individuals without insurance may pay the pharmacy the full price of the drug, as advertised by the pharmaceutical company.

On the surface, patient assistance programs would appear to be a mechanism through which drug companies, acting altruistically, can ensure that critical drugs are made available at affordable prices to patients who need them. Beneath the surface, however, the Committee

⁵⁸ See Stephanie Marken, *U.S. Uninsured Rate 11.9% in Fourth Quarter of 2015*, Gallup (Jan. 7, 2016), found at, <http://www.gallup.com/poll/188045/uninsured-rate-fourth-quarter-2015.aspx> (last visited Dec. 5, 2016).

⁵⁹ See Robert P. Navarro and Rusty Hailey, *Overview of Prescription Drug Benefits in Managed Care*, 19, in Robert Navarro, *Managed Care Pharmacy Practice* (2d Ed. 2009).

⁶⁰ See Congressional Budget Office, *Prescription Drug Pricing in the Private Sector*, at 12 (Jan. 1, 2007).

⁶¹ *Id.*

⁶² See Philip E. Johnson, *Patient Assistance Programs and Patient Advocacy Foundations: Alternatives for Obtaining Prescription Medications When Insurance Fails*, 64 Am. J. of Health-Sys. Pharmacy, S13, S13 (Nov. 1, 2006).

⁶³ See Marie A. Chisholm and Joseph T. DiPiro, *Pharmaceutical Manufacturer Assistance Programs*, 7 Arch Intern Med, 780, 780 (Apr. 8, 2002).

⁶⁴ See Benjamin Elgin and Robert Langreth, *How Big Pharma Uses Charity Programs to Cover for Drug Price Hikes*, Bloomberg Business Week (May 19, 2016), found at, <https://www.bloomberg.com/news/articles/2016-05-19/the-real-reason-big-pharma-wants-to-help-pay-for-your-prescription> (last visited Dec. 11, 2016).

⁶⁵ *Id.*

⁶⁶ See Marie A. Chisholm and Joseph T. DiPiro, *Pharmaceutical Manufacturer Assistance Programs*, 7 Arch Intern. Med., 780, 781 (Apr. 8, 2002).

⁶⁷ *Id.*

⁶⁸ *Id.*

⁶⁹ Philip E. Johnson, *Patient Assistance Programs and Patient Advocacy Foundations: Alternatives for Obtaining Prescription Medications When Insurance Fails*, 64 Am. J. of Health-Sys. Pharmacy, S13, S13 (Nov. 1, 2006).

found that self-serving motives are critical to understanding why some of the companies that were the subject of this investigation established these programs and structured them as they did. For example, as is described in greater detail below, internal documents show that Valeant viewed its patient assistance program—dubbed the “Valeant Coverage Plus Program” (“VCP”)—as a means to “maximize patient acquisition and retention,” and “enhance per patient value” for the company.⁷⁰ Valeant understood that the “Copay Cards” it made available through VCP to privately-insured individuals would reduce their incentive to complain to the press about Valeant’s outrageous price increases.⁷¹ Valeant even described its VCP as a “key marketing initiative.”⁷²

VCP demonstrates how a company can use a patient assistance program to erode competitive market pressure by subsidizing purchases of its own products. In the context of federal health insurance programs such as Medicare, Congress has prohibited such strategies through the federal Anti-Kickback Act, passed in 1972.⁷³ The Anti-Kickback Act bars individuals or entities from offering, paying, soliciting, or receiving remuneration in order to obtain any business that is reimbursed under the Medicare program, state health care programs, or other applicable federal programs. Many experts have praised the Act, noting that removing kickbacks promotes a more effective and better functioning market.⁷⁴

There are also a number of government programs that help keep the price of drugs low for certain entities. Many non-profit health care providers that serve safety-net populations also get government 340B program pricing that requires manufacturers to provide their drugs at a reduced price.⁷⁵ Medicare, Medicaid, and the Department of Defense and Department (“DOD”) of Veterans Affairs (“VA”) also pay reduced prices from the retail price for prescription drugs per statute.⁷⁶

⁷⁰ See, *infra*, at 58.

⁷¹ *Id.*

⁷² *Id.*

⁷³ See 42 U.S.C. 1320A-7B. Valeant does not offer copay assistance to individuals who are insured through Medicare. See Deposition of J. Michael Pearson, at 27:19–30:21 (Apr. 18, 2016) (“Pearson Deposition”).

⁷⁴ See, e.g., David H. Howard, *Drug Companies’ Patient-Assistance Programs—Helping Patients or Profits?* 371 *New England Journal of Medicine*, 97, 99 (July 10, 2014).

⁷⁵ The Health Resources and Services Administration calculates a 340B ceiling price for each covered outpatient drug. On average, hospitals in the 340B program receive a minimum discount of 22.5% of the average sales price for drugs. See MedPAC, *Overview of the 340B Drug Pricing Program*, vii (May 2015).

⁷⁶ Congress created the 340B program in November 1992. It is codified as Section 340B of the Public Health Service Act (created under the Veterans Health Care Act of 1992). The law gives certain clinics and hospitals access to reduced prices on drugs. Medicare pays 106% of the Average Sales Price for each drug, which is calculated by each manufacturer inclusive of rebates. For Medicaid, the rebate for drugs varies, but for most of them, manufacturers must provide at least a 13% rebate discount to Medicaid programs. DOD and VA have their own negotiated prices for drugs with at least a 24% discount from the non-Federal average manufacturer price. See Alison Mitchell, *Centers for Medicare & Medicaid Services: President’s FY2015 Budget*, Congressional Research Service, pages 17, 21 (May 15, 2014), *found at*, <https://fas.org/sgp/crs/misc/R43446.pdf> (last visited Dec. 11, 2016); U.S. Dep’t of Health & Human Servs., *Medicare Part B Reimbursement of Prescription Drugs*, (Jun. 2014), *found at*, <https://aspe.hhs.gov/report/medicare-part-b-reimbursement-prescription-drugs> (last visited Dec. 7, 2015); Center for Medicaid and CHIP Services, *Medicaid Drug Rebate Program*, *found at*, <http://www.medicaid.gov/medicaid-chip-program-information/by-topics/benefits/prescription-drugs/medicaid-drug-rebate-program.html> (last visited

Distribution Structure. Most prescription drugs are distributed through retail or mail order pharmacies of a customer's choosing or as determined by a customer's insurance policy. For certain drugs, however, there is a separate distribution structure through specialty pharmacies.

Specialty pharmacies typically distribute specialty drugs and perform a number of other services, which may include helping to administer complex drugs that must be infused or that can have serious side effects, performing patient education, and monitoring patients' reactions to prescribed medications (*e.g.* chemotherapy drugs). In addition, specialty pharmacies may handle paperwork associated with insurer reimbursement, manufacturer data reporting, and FDA reporting requirements. There is currently no industry standard for what qualifies as a specialty drug.

In some cases, manufacturers sell drugs in exclusive or limited networks that only allow dispensing from one or more specialty pharmacies. Traditionally, this type of limited distribution network involves drugs that require specific and complex dosing or lab monitoring. Sometimes the FDA also predicates drug approval on specialty pharmacy distribution for these reasons. For example, the *Food and Drug Administration Amendments Act of 2007* reserved the right of the FDA to order Risk Evaluation and Mitigation Strategies ("REMS") for the approval of drugs with increased toxicity and risk factors.⁷⁷ These drugs are sold in specialty pharmacies where pharmacists are trained to follow special dosing and storage requirements, conduct continual lab monitoring, and maintain safety protocols. In this scenario, by restricting access to a drug, the FDA and a manufacturer can ensure that patients only receive the drug from specialty pharmacies that have been trained on the necessary monitoring to reduce risks.

Recently, however, some manufacturers have begun to use specialty pharmacies as a way to increase sales instead of for the traditional uses discussed above.

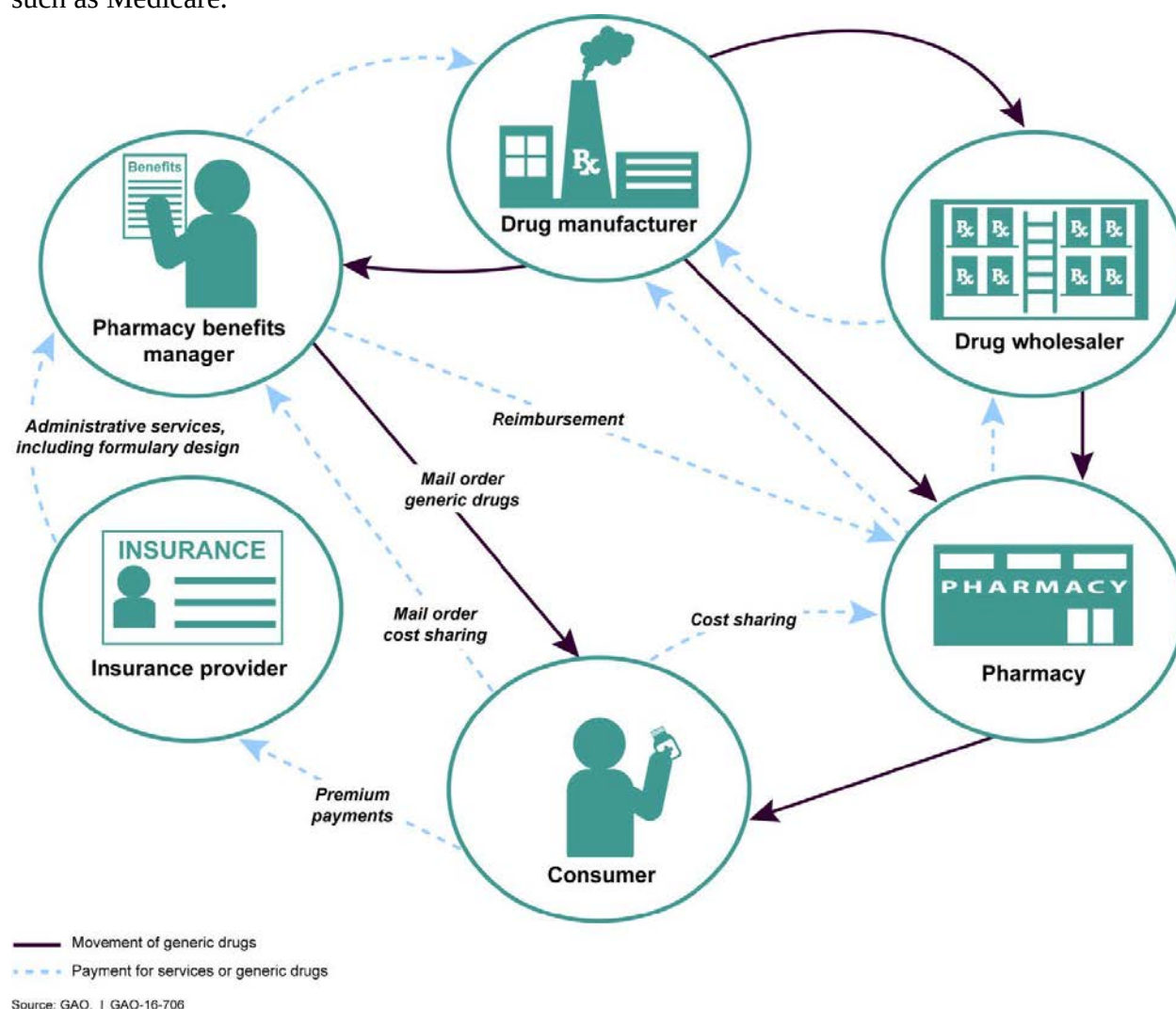
As a result, specialty pharmacies today sell a wide range of drugs often at steep prices, including drugs to treat conditions such as toenail fungus and acne that do not meet the traditional specialty drug criterion discussed above. In 2015, drugs sold in specialty pharmacies represented one to two percent of prescriptions yet these drugs accounted for more than 38 percent of drug spending.⁷⁸

Dec. 7, 2015); *Comparison of DOD and VA Direct Purchase Prices*, The Government Accountability Office, GAO-13-358, at 2 (Apr. 2013).

⁷⁷ 121 Stat. 823 (2007), *codified at*, 21 U.S.C. § 355-1.

⁷⁸ See The Pew Charitable Trusts, *Fact Sheet: Specialty Drugs and Health Care Costs* (Nov. 16, 2016), *found at*, <http://www.pewtrusts.org/en/research-and-analysis/fact-sheets/2015/11/specialty-drugs-and-health-care-costs> (last visited Dec. 6, 2016).

Figure 1. Structure of Drug Industry. Note that insurance provider includes government payers, such as Medicare.



III. FDA Regulation of Pharmaceuticals

The FDA, within the Department of Health and Human Services (“HHS”), oversees the approval and regulation of drugs entering the U.S. market. The FDA’s regulatory responsibilities include the safety and effectiveness of potential entries into the market, both for new innovator products and for generic products—copies of approved drugs, which are formulations chemically and biologically equivalent to already approved drugs.⁷⁹ The agency also has post-approval regulatory responsibilities.

⁷⁹ The FDA defines a generic drug as one that is biologically equivalent to another drug product in dosage form, strength, route of administration, quality, and intended use. Committee Staff Briefing with the FDA (Nov. 20, 2015) (“November 2015 FDA Briefing”).

A. New Drug Applications

To obtain FDA approval for a new drug, a sponsor submits a New Drug Application (“NDA”) containing data on the safety and effectiveness of the drug as determined through clinical trials and other research for FDA review.⁸⁰ Compiling this evidence involves several discrete steps, from preclinical testing to small scale and then larger scale human testing.⁸¹ Following human clinical testing, some sponsors will formally file an NDA—which includes findings from all tests as well as data on how the product is manufactured—asking the FDA to approve the drug to be marketed in the United States.⁸² The FDA has 60 days from submission of an application to determine whether it can be filed for review and assigned to a review team.⁸³

B. Abbreviated New Drug Applications

The FDA process for approving generic drugs is more streamlined compared to the NDA process. Generic drugs are required to submit an Abbreviated New Drug Application (“ANDA”) to demonstrate equivalence to a product the FDA has already approved. This application allows a company to make use of the patented drug’s existing safety and efficacy data already on file with the FDA, and the generic drugs are generally not required to include animal and human testing data to establish safety and effectiveness. Instead, a generic applicant must scientifically demonstrate that its product is bioequivalent (*i.e.*, performs in the same manner as the innovator drug).⁸⁴ One way to demonstrate bioequivalence is to measure the rate of absorption of the generic drug, the time it takes to reach the bloodstream, in 24 to 36 healthy volunteers.⁸⁵ The generic version must have the same rate of absorption as the innovator drug, indicating that it delivers the same amount of active ingredients into a patient’s bloodstream in the same amount of time as the innovator drug.⁸⁶ In addition to determining bioequivalence of a proposed generic product, the FDA reviews manufacturing facilities and drug labeling information prior to granting approval.⁸⁷ Generally, ANDA reviews consist of data review, but the FDA has the discretion to (and sometimes does) run tests to confirm the accuracy of data in ANDAs.⁸⁸

As part of its oversight of drug manufacturing, the FDA also has requirements specific to the manufacturing of active pharmaceutical ingredients (“API”), which are in addition to its

⁸⁰ *Id.*

⁸¹ *Id.*

⁸² *Id.*

⁸³ See FDA, *Drug Approval Process* (undated), found at,

<http://www.fda.gov/downloads/drugs/resourcesforyou/consumers/ucm284393.pdf> (last visited Dec. 9, 2016).

⁸⁴ November 2015 FDA Briefing. Using bioequivalence as the basis for approving generic copies of drug products was established by the *Drug Price Competition and Patent Term Restoration Act of 1984*, also known as the Hatch-Waxman Act. 98 Stat. 1585 (1984), codified in provisions of 21 U.S.C. This Act, among other provisions, expedites the availability of less costly generic drugs by permitting FDA to approve applications to market generic versions of brand-name drugs without conducting costly and duplicative clinical trials. See generally, *id.*

⁸⁵ See U.S. FDA, *Abbreviated New Drug Application (ANDA): Generics* (Nov. 16, 2016), found at, <http://www.fda.gov/Drugs/DevelopmentApprovalProcess/HowDrugsareDevelopedandApproved/ApprovalApplications/AbbreviatedNewDrugApplicationANDAGenerics/> (last visited Dec. 13, 2016).

⁸⁶ *Id.*

⁸⁷ November 2015 FDA Briefing.

⁸⁸ *Id.*

guidance for the manufacture of finished drug products.⁸⁹ The requirements include quality review programs, personnel, manufacturing facilities, and distribution procedures, among others. The FDA maintains a list of facilities that meet the requirements.⁹⁰

C. Controls on Importation

All drugs imported into the United States require FDA approval, and that approval is granted only to U.S. pharmacists and wholesalers.⁹¹ In 1954, the FDA issued a personal use exemption that allows individuals to bring up to a 90-day supply of a drug in to the United States under certain conditions their personal use.⁹² The FDA has also used the personal use exemption to allow imports in other cases, including the importing of new AIDS drugs in 1988.⁹³ In addition, in 2003, Congress passed the *Medicare Prescription Drug, Improvement, and Modernization Act* (“MMA”).⁹⁴ This law, in addition to implementing Medicare Part D, permits limited importation of certain drugs from Canada, provided the Secretary of Health and Human Services first certifies that those drugs are safe and that the program would lower costs for U.S. consumers.⁹⁵ To the Committee’s knowledge, no HHS Secretary has ever taken this step. The FDA has identified concerns with drug safety as a primary reason for not exercising this authority.⁹⁶ Others have raised concerns that importation of foreign drugs could stifle U.S.

⁸⁹ November 2015 FDA Briefing. The FDA, in resources for industry, further defines an API as:

[A]ny substance that is represented for use in a drug and that, when used in the manufacturing, processing, or packaging of a drug, becomes an active ingredient or a finished dosage form of the drug. Such substances are intended to furnish pharmacological activity or other direct effect in the diagnosis, cure, mitigation, treatment or prevention of disease, or to affect the structure and function of the body of humans or other animals.

FDA, *For the Industry: What Must I do to Import a Human Drug Product That Has Been Approved by the FDA Into the United States* (undated), found at, <http://www.fda.gov/ForIndustry/FDABasicsforIndustry/ucm238032.htm> (last visited Dec. 10, 2016).

⁹⁰ November 2015 FDA Briefing. The list, which also includes some facilities that produce only Finished Dosage Forms (a different intermediate product) includes 2,515 facilities, of which about 70 percent are outside the United States. India is the largest producer outside the United States, followed by China, Italy, Germany, and Canada. See GMP News, *FDA Publishes List of GMP Facilities Producing for the US Market (Generic Drug APIs)* (Oct. 24, 2013), found at, http://www.gmp-compliance.org/enews_03940_FDA-publishes-List-of-GMP-facilities-producing-for-the-US-market--generic-drug-products-and-APIs-.html (last visited Dec. 10, 2016).

⁹¹ 21 U.S.C. §331 prohibits the importation of unapproved drugs.

⁹² See Peter S. Reichertz & Melinda S. Friend, *Hiding Behind Agency Discretion: The Food and Drug Administration’s Personal Use Drug Importation Policy*, 9 Cornell J.L. & Pub. Pol’y, 493, 513 (2000).

⁹³ *Id.* at 500.

⁹⁴ 117 Stat. 2066 (2003).

⁹⁵ *Id.* at 2464–69.

⁹⁶ See *Importation of Prescription Drugs: Hearing Before S. Comm. on Health, Educ., Labor, and Pensions*, 108th Cong., 2d Sess., S. Hrg. 108–470, at 11–12 (May 20, 2004) (testimony of John M. Taylor, FDA); Some States have attempted to allow importation of certain drugs from certain countries. For example, in 2013 Maine passed amendments to the Maine Pharmacy Act (32 M.R.S. §§ 13701–13847), which allowed its residents to import prescription drugs through a broker from licensed pharmacies in Australia, Canada, New Zealand and the United Kingdom. See 2013 Me. Legis. Serv. Ch. 373 (S.P. 60) (L.D. 171). In 2015, the U.S. District Court ruled that the law was invalid, because it was preempted by federal laws prohibiting such importation. See *Ouellette v. Mills*, 91 F. Supp. 3d. 1, 8–12 (D. Me. 2015).

innovation.⁹⁷ Some scholars maintain that several other countries have regulatory regimes similar to those of the United States, which ought to mitigate quality concerns.⁹⁸

Using the principle of “enforcement discretion,” however, the FDA in the past has approved the temporary importation of foreign drugs in cases when there is a shortage of an approved U.S. drug that is critical to patients, if the shortage cannot be resolved by manufacturers of the approved U.S. drug in the immediate future.⁹⁹ In these cases, FDA searches for similar products approved in foreign markets that may help meet critical patient needs in the United States.¹⁰⁰ The FDA identifies the product, evaluates it and its manufacturing chain for quality and safety, and ensures that the manufacturer is willing and able to import the drug.¹⁰¹ The FDA’s exercise of this enforcement discretion can provide a supply of foreign drugs to the United States during a critical medical shortage; however, this license is temporary, and is not equivalent to attaining FDA approval for marketing in the United States.¹⁰² According to FDA estimates, the agency uses such enforcement discretion extremely sparingly—deploying it in about five percent of drug shortage cases.¹⁰³ Notable instances include the 2012 importation of a substitute for Johnson & Johnson’s Doxil manufactured by an Indian generic company; the 2012 license to Hospira to import methotrexate from one of its Canadian facilities; and the 2013 importation of total parenteral nutrition drugs.¹⁰⁴

D. FDA Approval for Generics

1. Generic Approval Times

While FDA requirements for the approval of generic drugs is streamlined relative to the requirements for new drug entities, the process is still lengthy. Median times from ANDA application submission to approval was 36 months in 2013, rising to 43 months in 2014 and 48 months in 2015.¹⁰⁵ Currently, more than half of the applications for generic entrants take four years or more to attain approval. The FDA has attributed these long approval periods to

⁹⁷ Committee Staff interviews with compendium of experts from the following institutions: Duke University, Georgetown University, Harvard University (Aaron Kesselheim and others), Johns Hopkins University (Gerard Anderson, Joshua Sharfstein, and others), Massachusetts Institute of Technology, University of California—Berkeley, University of Chicago, University of Minnesota, July—December, 2016 (“Expert Compendium”) Expert Compendium.

⁹⁸ *Id.*

⁹⁹ See FDA, *Frequently Asked Questions: Temporary Importation of Lipodox*, at 1–2 (undated), found at, <http://www.fda.gov/downloads/Drugs/DrugSafety/DrugShortages/UCM295225.pdf> (last visited Dec. 6, 2016); Committee Staff Briefing with FDA (Dec. 17, 2015) (“December 2015 FDA Briefing”).

¹⁰⁰ December 2015 FDA Briefing.

¹⁰¹ *Id.*

¹⁰² *Id.*

¹⁰³ FDA, *About FDA: Executive Summary: A Review of FDA’s Approach to Medical Product Shortages* (undated), found at, <http://www.fda.gov/AboutFDA/ReportsManualsForms/Reports/ucm277744.htm> (last visited Dec. 7, 2016).

¹⁰⁴ See Alexander Gaffney, *Regulatory Affairs Professional Society, Facing Dire Shortage of IV Saline, FDA Again Turns to Enforcement Discretion Approach* (Apr. 30, 2014), found at, <http://www.raps.org/focus-online/news/news-article-view/article/4933/> (last visited Dec. 6, 2016).

¹⁰⁵ See FDA, *Industry and FDA Overview on GDUFA: PDA/FDA Join Regulatory Conference*, at 16 (Sept. 29, 2015), found at, <http://www.fda.gov/downloads/ForIndustry/UserFees/GenericDrugUserFees/UCM470981.pdf> (last visited Dec. 6, 2016).

increases in annual ANDA submissions, including a growing number of foreign facilities making generic drugs, that has outpaced the increase in the FDA’s resources for generic review and oversight, as well as the time it has taken to clear out a historical backlog of applications.¹⁰⁶

2. Application Fees for Generic Drugs

Generic applicants can face FDA fees well in excess of \$70,000. The phrase generic drug submission can refer to an ANDA, an amendment to an ANDA, or a prior approval supplement to an ANDA, and each process carries a separate fee. For Fiscal Year (“FY”) 2017, the ANDA fee is \$70,480, and the prior approval supplement fee is \$35,240.¹⁰⁷ These fees are due on the date of submission of the application. Generic drug applicants also pay other fees ranging from \$44,234 to \$59,234 for the use of approved API facilities. Lastly, each API facility must pay an annual fee to the FDA to remain approved.¹⁰⁸ The cost of bringing new novel drugs to the market is, of course, substantially higher. A 2015 analysis estimated that after-tax costs for research and development plus pre-and post-approval expenditures (including FDA fees) averaged about \$1 billion for drugs introduced in the United States from 2005 through 2009.¹⁰⁹

Long approval times and lack of information on applications add uncertainty to the business calculation of whether to enter the market with a generic drug. While the FDA has taken steps to improve transparency related to certain aspects of its oversight process, information on ANDA applications submitted to the FDA and on the status of those applications is not publicly available due to potential trade secret and securities concerns.¹¹⁰ Thus, a potential generic drug applicant does not know whether other drug companies have filed applications or what the status of those applications may be. Some drug companies hire consultants to uncover any available information, without guarantees of reliability. Some stakeholders and experts have maintained that the lack of information on potential competitor entry makes it difficult for industry to predict FDA timing and to make informed decisions regarding their own entry. For example, knowing whether a company is likely to be the second generic entrant or the seventh could affect a company’s entry decision.

The FDA has taken steps to improve transparency related to certain aspects of its oversight process, which improves its visibility into the drug supply chain and facilitates decision-making by generic entrants. For example, FDA databases now include more accurate information on facilities involved in the manufacture of drugs, with more than 3,900 facilities

¹⁰⁶ Committee Staff Briefing with FDA (Aug. 2, 2016) (“August 2016 FDA Briefing”). In 2012, the FDA received 1103 ANDA submissions; in 2000, it received 335. *Id.* See, *infra*, at 110 for information on legislation enacted to address this backlog.

¹⁰⁷ See FDA, *Abbreviated New Drug Application (ANDA) and Prior Approval Supplement (PAS) Fees*, (Aug. 1, 2016),

found at, <http://www.fda.gov/ForIndustry/UserFees/ucm319568.htm> (last visited Dec. 15, 2016).

¹⁰⁸ See FDA, *Generic Drug User Fee Amendments of 2012: Questions and Answers Related to User Fee Assessments*, at 3 (Nov. 2016), found at, <http://www.fda.gov/downloads/drugs/guidancecomplianceregulatoryinformation/guidances/ucm316671.pdf> (last visited Dec. 15, 2016).

¹⁰⁹ See Ernst R. Berndt, et.al. *Decline in Economic Returns from New Drugs Raises Questions About Sustaining Innovations*, Health Affairs, at 249 (Feb. 2015).

¹¹⁰ November 2015 FDA Briefing.

that support generic drug applications self-identifying each year.¹¹¹ Nevertheless, since information on ANDA applications is not available, potential entrants do not have access to information regarding the number of parallel applicants or approval timelines, making it difficult for industry to predict the FDA's timing and subsequent competition to make informed decisions regarding entry.

3. Generic Application Backlogs

In 2012, Congress sought to speed the process for approving safe and effective generic drugs by authorizing a new user fee program for generic drugs under the *Food and Drug Administration Safety and Innovation Act* ("FDASIA").¹¹² The *Generic Drug User Fee Amendments* ("GDUFA"), which became effective October 1, 2012, and will sunset at the end of a five-year period, established a user fee program for generic drugs.¹¹³ Under GDUFA, the FDA collects fees from drug companies at certain stages of the generic drug application and approval process and can use the additional funds for activities such as reviewing submissions, issuing approvals, and monitoring and inspections, among other activities.¹¹⁴ The law defines the specific generic drug activities for which the FDA can use the funds, including review of submissions, issuance of approvals, inspections and monitoring, and other activities.¹¹⁵

GDUFA linked the continued fee increases to FDA performance requirements, set to commence in the later years of the program. One requirement concerned a backlog of applications received prior to October 2012, which included 2,866 ANDAs and 1,873 prior approval supplements. The FDA announced in July 2016 that it had acted on more than 90 percent of those backlogged submissions, ahead of the September 30, 2017, deadline set forth in the Act.¹¹⁶ ANDAs submitted in FY 2016 have a GDUFA first-action goal date of 15 months and those submitted in FY 2017 will receive a 10 month GDUFA goal date.¹¹⁷ Some GDUFA requirements are just beginning to come due at the printing of this Report, and the FDA has stated it has met performance goals for ANDAs submitted after the start of the GDUFA program. The agency expects to eliminate the backlog of ANDAs by the next re-authorization of GDUFA in 2017.¹¹⁸

¹¹¹ See FDA, *Industry and FDA Overview on GDUFA: PDA/FDA Join Regulatory Conference*, at 34 (Sept. 29, 2015), found at, <http://www.fda.gov/downloads/ForIndustry/UserFees/GenericDrugUserFees/UCM470981.pdf> (last visited Dec. 6, 2016).

¹¹² 126 Stat. 993 (2012).

¹¹³ 126 Stat. 993 (2012).

¹¹⁴ See generally, *id.*

¹¹⁵ See generally, 126 Stat. 993 (2012). The law also provides for streamlined hiring authority for FDA positions, and links the program to requirements for annual performance and spending reports by the FDA. *Id.* These provisions are authorized from October 1, 2012 and expire September 30, 2017 unless reauthorized. *Id.*

¹¹⁶ See *Prioritizing Public Health: The FDA's Role in the Generic Drug Marketplace: Hearing Before the Subcomm. on Agriculture, Rural Development, Food and Drug Administration, and Related Agencies of the S. Appropriations Comm.*, at 5 (Sept. 21, 2016) (written testimony of Hon. Janet Woodcock M.D.).

¹¹⁷ *Id.* at 6.

¹¹⁸ See Zachary Brennan, *FDA's Woodcock: Generic Drug Application Will be Eliminated Before GDUFA II*, Regulatory Affairs Prof'l Soc'y, (Jan. 28, 2016), found at, <http://www.raps.org/Regulatory-Focus/News/2016/01/28/24195/FDA%E2%80%99s-Woodcock-Generic-Drug-Application-Backlog-Will-be-Eliminated-Before-GDUFA-II/> (last visited Dec. 6, 2016).

IV. Increasing Drug Prices

A. Prescription Drug Pricing Trends

After a period of relatively stable prices, spending on retail prescription drugs has been rising. In 2014, this spending rose in real terms by 12.2 percent, the largest increase since 2002.¹¹⁹ Broader measures that include hospital and other prescription drug spending increased by 11.5 percent.¹²⁰ In contrast, over the previous nine years, retail prescription drug spending growth had averaged 1.8 percent a year.¹²¹ The introduction of new high-priced brand name drugs, and price increases in existing branded drugs have contributed to this increase pattern. The Centers for Medicare and Medicaid (“CMS”) recently reported that the drugs on which Medicare Part D plans spent the most are (1) older drugs with the highest claims counts, such as Lisinopril, used to treat high blood pressure, and levothyroxine sodium, which treats hypothyroidism and (2) brand name high-profile drugs introduced more recently, such as the drug Sovaldi, used to treat hepatitis C, and Revlimid, a cancer drug.¹²²

B. Large Price Spikes for Off-Patent Drugs

Examples of sharp rises in the cost of off-patent drugs are being reported with increasing frequency. In addition to price hikes by the four companies that were the focus of the Committee’s investigation, other drugs have also garnered attention. In April 2016, a study found that the mean price of insulin, a lifeline therapy for the 29 million Americans with diabetes, increased from \$4.34 per milliliter in 2002 to \$12.92 per milliliter in 2013, a 200 percent increase.¹²³ In July 2016, a flurry of news outlets reported another staggering price spike: the price of Naloxone, the antidote to prescription painkiller overdoses, increased by 1,000 percent, amidst an opioid public health crisis.¹²⁴ In August 2016, Chairman Collins and Ranking Member McCaskill wrote to the CEO of Mylan requesting answers about the

¹¹⁹ See Lucy Lerner, *Health Affairs Web First: National Health Spending Growth Accelerates in 2014*, Health Affairs, (Dec. 2, 2015), found at, <http://healthaffairs.org/blog/2015/12/02/health-affairs-web-first-national-health-spending-growth-accelerates-in-2014/> (last visited Dec. 2016).

¹²⁰ [UNDER SEAL] (on file with Committee).

¹²¹ *Id.*

¹²² See CMS, *Updated Prescriber-Level Medicare Data*, (Aug. 18, 2016), found at, <https://www.cms.gov/Newsroom/MediaReleaseDatabase/Fact-sheets/2016-Fact-sheets-items/2016-08-18.html> (last visited Dec. 6, 2016). Abilify, Crestor, and Spivera are among the top 10 in Medicare Part D spending for 2014. *Id.*

¹²³ See Xinyang Hua, Natalie Carvalho, Michelle Tew, Elbert Huang, William Herman, Philip Clarke, *Expenditures and Prices of Antihyperglycemic Medications in the United States: 2002-2013*, 315 J. of the Am. Med. Assoc. 1400, 1401 (Apr. 5, 2016). Some insulin is on patent, but some is not.

¹²⁴ See Melody Petersen, *Narcan Prices Increase “By 1000 Percent or More”*, Los Angeles Times (July 20, 2016), found at, <http://www.ems1.com/health-and-wellness/articles/109046048-Narcan-prices-increase-by-1000-percent-or-more/> (last visited Dec. 6, 2016). Some Naloxone administration devices are on patent—naloxone is not. See Joshua Lloyd (FDA), *The Clinical Use of Naloxone*, FDA, at3, found at, <http://www.fda.gov/downloads/Drugs/NewsEvents/UCM454748.pdf> (last visited Dec. 12, 2016).

company's 500 percent price spike in its epinephrine auto-injector, EpiPen, used to save lives during an allergic emergency.¹²⁵

The detailed analysis in a recent U.S. Government Accountability Office ("GAO") study on generic drug price trends supports the perception of widespread price spikes.¹²⁶ More than 300 of the 1,441 established generic drugs that the GAO examined had one or more instances of "extraordinary price increases," defined as periods of prices at least doubling over the five-year study period.¹²⁷ In 2014 (and the first quarter of 2015), more than 100 generic drugs experienced these extraordinary increases in price.¹²⁸ For 48 of the drugs, the extraordinary price increases were 500 percent or higher.¹²⁹ Nearly all of the drugs with extraordinary price increases maintained those higher prices for at least the next year, and continued to persist for those drugs where the data allowed further tracking.¹³⁰

Although generic drugs continue to be a source of significant cost savings for the U.S. health care system overall, these savings are beginning to be eroded by the steep price spikes on this relatively small number of generic drugs.¹³¹

The GAO also found that competition "is the primary driver of generic drug prices," and "less competition could drive prices higher."¹³² While many factors can reduce competition in the generic drug market, the GAO noted that leading factors are consolidation among manufacturers or purchasers of a drug, lack of access to a drug's Active Pharmaceutical Ingredient ("API"), and the fact that a drug serves a small patient population.¹³³ The GAO also noted that competition could be increased by clearing out the backlog in the FDA generic approval process.¹³⁴

C. Generic Price Increases

For several decades, generic drugs provided relief from rising prescription drug prices with the increased availability and usage of lower-cost generic versions of branded drugs. According to the Generic Pharmaceutical Association, generic drugs now make up 89 percent of prescriptions dispensed in the United States.¹³⁵ Generic drugs saved Americans \$227 billion in

¹²⁵ See Letter from the Hon. Susan M. Collins & the Hon. Claire McCaskill to Heather Bresch, CEO Mylan, Inc. (Aug. 24, 2016). While EpiPen®'s delivery system is patented, epinephrine is not. See Andrew Pollack, *Mylan Raised EpiPen's Price Before the Expected Arrival of a Generic*, N.Y. Times (Aug. 24, 2016).

¹²⁶ See U.S. Gov't Accountability Office, *Generic Drugs Under Medicare*, GAO-16-706 (Aug. 2016).

¹²⁷ *Id.* at 3, 12.

¹²⁸ *Id.* at 12.

¹²⁹ *Id.* at 14.

¹³⁰ *Id.* at 17.

¹³¹ It is worth noting that few of the drugs identified by the GAO as experiencing extraordinary price increases were among the top 100 generic drugs used in the Medicare Part D program (*Id.* at 18).

¹³² *Id.* at Highlights.

¹³³ *Id.* at 23-24.

¹³⁴ *Id.* at 26.

¹³⁵ See Quintile IMS Health report, *2016 Generic Drug Savings and Access in the United States Report*, Generic Pharmaceutical Assoc. at 5, found at, <http://www.gphaonline.org/media/generic-drug-savings-2016/index.html> (last

2015.¹³⁶ With the introduction of a generic version of drugs as the patents of branded drugs expired, prices would fall and often continue to fall over time as additional generics entered.¹³⁷ These decreases have partly offset increases in the overall costs of prescription drugs.

Competition is the primary driver of drug prices.¹³⁸ Generic producers set prices of their multi-source products based on the price at which the drug is currently being sold in the market, with new entrants often setting a price somewhat lower. According to manufacturers, the market for generic drugs operates in a sense like a commodities market, with companies being asked to submit their best price to their customers—pharmacy organizations or wholesalers. The greater the number of manufacturers, the lower the price generally is. According to a 2016 IMS Institute for Healthcare Informatics report, the immediate price reduction in the cost of drugs following generic entry is substantial and is followed by continued savings in subsequent years.¹³⁹ Similarly, declines in the number of generic manufacturers for a drug often result in price increases.¹⁴⁰ According to industry stakeholders and several analyses, manufacturer consolidation through acquisition has contributed to higher prices.¹⁴¹

visited Dec. 16, 2016). The report notes that nearly 3.9 billion of the total 4.4 billion prescriptions dispensed in the U.S. in 2015 were filled using generic drugs.

¹³⁶ *Id.* at 4.

¹³⁷ *Id.* at 4.

¹³⁸ See U.S. Gov't Accountability Office, *Generic Drugs Under Medicare*, at 23, GAO-16-706 (Aug. 2016).

¹³⁹ See IMS Institute for Healthcare Informatics, *Price Declines after Branded Medicines Lose Exclusivity in the U.S.*, at 2 (Jan. 2016), found at <https://www.imshealth.com/files/web/IMSH%20Institute/Healthcare%20Briefs/PhRMA%20Generic%20Price%20Brief%20January%202016.pdf> (last visited Dec. 6, 2016).

¹⁴⁰ *Id.*

¹⁴¹ See Trefis Team, *Why Are Generic Drug Prices Shooting Up?* Forbes (Feb. 27, 2015) found at <http://www.forbes.com/sites/greatspeculations/2015/02/27/why-are-generic-drug-prices-shooting-up/#3438fe3d377e> (last visited Dec. 7, 2016); RM Conti, MB Landrum, and M Jacobson, *The Impact of Provider Consolidation on Outpatient Prescription Drug-Based Cancer Care Spending*, at 1 The Health Cost Institute (undated).

CHAPTER 2. THE BUSINESS MODEL

The Committee found that each of the four companies that were the subjects of this investigation followed a business model that enabled them to identify and acquire off-patent drugs over which they could exercise de facto monopoly pricing power due to a market failure. The companies then imposed (and protected) astronomical price increases. Internal documents show that the companies paid careful attention to the business model and even presented analyses to potential investors describing how well the drugs they had targeted fit each aspect of the model. With some variation, the business model employed by the companies that were the subject of the Committee’s investigation consists of the five central elements described below.

1. Sole-Source. The first element of the business model was to acquire a sole-source drug, for which there was only one manufacturer. By definition, a company that controlled access to a sole-source drug faced no immediate competition and could therefore price the drug aggressively. Once competitors enter the market, that pricing power declines rapidly. Studies show that generic competition greatly reduces the price of drugs, typically by about 50 percent in the first year generics entered the market.¹⁴² Drugs with competition from three or more generics often face generic competition priced at just 25 percent of the brand name drug price, or even lower.¹⁴³ The Committee found that the length of time a sole-source drug was expected to have the field to itself was an important consideration to the companies.

2. Gold Standard. The second element of the business model is to ensure the target drug was considered the “gold standard” treatment—*i.e.*, the best drug available for the condition it treated. The companies believed that “gold standard” drugs would be more valuable due to the high likelihood that physicians would continue to prescribe the drug and be reluctant to prescribe an alternative, even if the price of the drug increased significantly. The Committee found that the companies expected (correctly in most instances) that physicians would make special efforts to ensure their patients could access “gold standard” drugs regardless of price, such as completing prior authorization forms required to secure reimbursement from insurers or helping patients obtain financial support through other sources.¹⁴⁴

3. Small Market. The third element of the business model was to select a drug that served relatively few patients and that generated low revenues at its pre-increase price level. Such “small market” drugs often did not attract competitors and for that reason, some companies that controlled them have been able to exercise a de facto monopoly power.¹⁴⁵ As one expert witness noted, larger generic companies seem less likely to seek entry into markets where a brand drug had less than \$100 million in annual sales.¹⁴⁶

¹⁴² See IMS Institute for Healthcare Informatics, *Price Declines after Branded Medicines Lose Exclusivity in the U.S.*, at 4 (Jan. 2016).

¹⁴³ December 2015 Hearing, at 1 (written testimony of Gerard Anderson, Ph.D.).

¹⁴⁴ See Email from Tina Ghorban to Nancy Retzlaff, TUR-SCA00030993 (Nov. 13, 2015), and accompanying attachment, Assessing the Market Potential for Sulfadiazine and Pyrimethamine, TUR-SCA00030993, at TUR-SCA00031002 and TUR-SCA00031019 (June 10, 2015).

¹⁴⁵ See HHS, The Assistant Secretary for Planning and Evaluation, *ASPE Issue Brief, Understanding Recent Trends in Generic Drug Prices*, at 11 (Jan. 27, 2015).

¹⁴⁶ See December 2015 Hearing, at 3 (written testimony of Gerard Anderson, Ph.D.).

The Committee also found that the companies it investigated assumed that “small market” drugs provided another attractive advantage—the patient population dependent upon them would be too small to organize an effective opposition to the price increase.¹⁴⁷

4. Closed Distribution. The fourth element of the business model was to control access to the drug through a closed distribution system, specialty pharmacy, or some other means. Although the Committee found some variation in how the companies approached this element of the model, each company took steps to make it more difficult for competitors to enter the market. Turing, for example, used a closed distribution system to keep generic companies from getting the supply of Daraprim needed to conduct bioequivalence tests on generic alternatives, which is required to obtain FDA approval.¹⁴⁸

Valeant, on the other hand, may have used a subtler approach: It established a patient assistance program that may have been designed to attract and retain high-value patients (those who had private insurance or who could pay cash) while excluding low-value patients (*e.g.*, those without insurance). By doing so, Valeant could potentially draw the profits available in the market to itself, leaving potential competitors with a reduced prospect of making a profit if they attempted to offer a generic alternative.

5. Price Gouge. The final element of the business model was the ultimate goal—to maximize profits by increasing prices as much as possible. Each of the companies investigated by the Committee dramatically increased the price of the target drugs they controlled over a very short period. Turing, for example, increased the price of Daraprim from \$13.50 a pill to \$750 a pill—an increase of more than 5,000 percent—literally overnight.¹⁴⁹ It is worth noting that all of the drugs had been off-patent for decades, and none of the four companies had invested a penny in Research & Development to create the drugs or to significantly improve them.

The business model employed by the four companies was also actively supported and promoted by their investors.

¹⁴⁷ See Email from Tina Ghorban to Nancy Retzlaff, TUR-SCA00030993 (Nov. 13, 2015), and accompanying attachment, Assessing the Market Potential for Sulfadiazine and Pyrimethamine, TUR-SCA00030993, at TUR-SCA00031020 (Jun. 10, 2015).

¹⁴⁸ See Deposition of Edwin Urrutia, at 211:10–11 (Mar. 8, 2016) (“[H]aving closed distribution can increase a product life cycle by preventing generics from potentially getting your referenced product”) (“Urrutia Deposition”).

¹⁴⁹ See Andrew Pollack, *Drug Goes From \$13.50 a Tablet to \$750, Overnight*, N.Y. Times (Sept. 20, 2015).

CHAPTER 3. CASE STUDIES

I. Turing Pharmaceuticals, LLC

A. Company Background

Turing is a U.S. subsidiary of a Swiss company with most of its operations in New York. The company was founded on February 24, 2015, by Mr. Shkreli, its largest shareholder, who served as its CEO until he resigned on December 18, 2015, after his federal indictment on securities fraud charges.¹⁵⁰ Mr. Shkreli resigned as a Director of the Board on February 10, 2016.¹⁵¹

Mr. Shkreli had previously founded and served as CEO of Retrophin and left that company in October 2014.¹⁵² He brought with him Ron Tilles, who had been a consultant for Retrophin and later became Turing's Interim CEO and Chairman of the Board, Edwin Urrutia, who became Turing's Interim Chief Financial Officer ("CFO"), and Michael Smith, who became Turing's Senior Director of Business Development. Mr. Shkreli and his former-Retrophin colleagues brought with them the business model described in Chapter 3 which they had tested and refined at Retrophin.

Mr. Tilles, Mr. Urrutia, and Mr. Smith were deposed by Committee staff, and testified under oath at the Committee's hearing on March 17, 2016. Howard Dorfman, who served as Turing's General Counsel from December 2014 until he was fired in August 2015 for what may have been retaliation for his internal opposition to the price increase, also testified.¹⁵³ The Committee also deposed Turing's former Communications Director, Craig Rothenberg. Another important member of the Turing team was Nancy Retzlaff, Turing's Chief Commercial Officer.

After issuing subpoenas to Turing, the Committee obtained and reviewed almost 400,000 pages of Turing documents. Unfortunately, this total may not include all relevant documents. The Committee's investigation established that Mr. Shkreli often worked at his home apartment, and accordingly, the committee subpoenaed Mr. Shkreli personally to ensure that any Turing documents in his personal possession were produced. Mr. Shkreli, however, invoked the Fifth Amendment in response to this subpoena and did not produce any documents.

B. Daraprim Background

Daraprim is a 62-year-old brand name drug with the API pyrimethamine. It is considered

¹⁵⁰ See Letter from Martin Shkreli to Ron. Tilles, TUR-SCA00288756 (Dec. 18, 2015); Indictment in *United States v. Shkreli*, No. 1:15-cr-00637 (KAM) (E.D.N.Y.) (ECF NO. 1).

¹⁵¹ See Letter from Martin Shkreli to Turing Board of Directors, TUR-SCA00288755 (Feb. 10, 2016).

¹⁵² See, *infra*, at 41.

¹⁵³ March 2016 Hearing, Trans. at 31:10–16 (**Chairman Collins:** And, how soon after you expressed your opposition to this price increase were you fired? **Mr. Dorfman:** Umm, certainly less than a month. I would say approximately two—two to three weeks. **Chairman Collins:** Were you fired for cause? **Mr. Dorfman:** No. I was told I was not fired for cause).

the gold standard treatment for toxoplasmosis, a serious disease that, if not effectively treated, can lead to brain and organ damage, blindness, and death.¹⁵⁴ Toxoplasmosis is caused by a single-celled parasite called *Toxoplasma gondii*.¹⁵⁵ This parasite's only known host is cats, which then can infect intermediate hosts such as plants, soil, water, other animals, or people.¹⁵⁶ According to the U.S. Centers for Disease Control and Prevention ("CDC"), more than 60 million people in the United States may be infected with the parasite.¹⁵⁷ Pregnant women infected by the parasites can transmit congenital toxoplasmosis to their unborn children.¹⁵⁸ Few people who are infected will display symptoms because a healthy person's immune system can typically suppress the parasite, which survives in the body only as a cyst.¹⁵⁹ Individuals with compromised immune systems, however, are at risk of developing severe toxoplasmosis.¹⁶⁰ Infants and immune-suppressed patients with toxoplasmosis must be treated, often in a matter of days to prevent mortality and morbidity.¹⁶¹ Daraprim is an anti-parasitic compound administered orally in tablet format and is highly effective against *Toxoplasma gondii*.¹⁶²

C. The Acquisition of Daraprim

In March 2015, Turing entered into negotiations with Impax Pharmaceuticals ("Impax") to purchase the U.S. based licensing rights to Daraprim.¹⁶³ Turing bought Daraprim from Impax on August 7, 2015, for \$55 million.¹⁶⁴ Turing then increased Daraprim's price from \$13.50 a pill to \$750 a pill—*that same day*.¹⁶⁵ According to press accounts, at that time, Daraprim was only sold in 100-count bottles. Accordingly, a single bottle of Daraprim went from \$1,350 a bottle to \$75,000 a bottle. On November 24, 2015, Turing rolled out a "price cut" that Mr. Shkreli had promoted for months.¹⁶⁶ Under this price cut, the price was cut by 50 percent for inpatient

¹⁵⁴ See December 2015 Hearing, at 2–3 (Written Testimony of David Kimberlin, M.D.); March 2016 Hearing, at 2–3 (written testimony of Adaora Adimora, M.D., MPH).

¹⁵⁵ See Centers for Disease Control, Parasites—Toxoplasmosis (Toxoplasma infection) Toxoplasmosis Frequently Asked Questions, *found at*, http://www.cdc.gov/parasites/toxoplasmosis/gen_info/faqs.html. (last visited Nov. 21, 2016).

¹⁵⁶ See Centers for Disease Control, Parasites—Toxoplasmosis (Toxoplasma infection) Epidemiology & Risk Factors, *found at* <http://www.cdc.gov/parasites/toxoplasmosis/epi.html> (last visited Nov. 21, 2016); December 2015 Hearing, at 2 (written testimony of David Kimberlin, M.D.).

¹⁵⁷ See Centers for Disease Control, Parasites—Toxoplasmosis (Toxoplasma infection) Toxoplasmosis Frequently Asked Questions, *found at*, http://www.cdc.gov/parasites/toxoplasmosis/gen_info/faqs.html. (last visited Nov. 21, 2016).

¹⁵⁸ See Center for Disease Control, Parasites—Toxoplasmosis (Toxoplasma infection), Pregnant Women, *found at* http://www.cdc.gov/parasites/toxoplasmosis/gen_info/pregnant.html (last visited Nov. 21, 2016).

¹⁵⁹ See Centers for Disease Control, Parasites—Toxoplasmosis (Toxoplasma infection), Treatment, *found at*, <http://www.cdc.gov/parasites/toxoplasmosis/treatment.html> (last visited Nov. 21, 2016).

¹⁶⁰ *Id.*

¹⁶¹ December 2015 Hearing, Trans. at 37:3–14 (testimony of David Kimberlin, M.D.).

¹⁶² *Id.* at 3 (Dec. 9, 2015) (written testimony of David Kimberlin, M.D.)

¹⁶³ Deposition of Ron Tilles, at 77:16–22 (Mar. 9, 2016) ("Tilles Deposition").

¹⁶⁴ *Developments in the Prescription Drug Market: Oversight: Hearing Before H. Comm. on Oversight and Gov't Reform Hearing*, at 2 (Feb. 4, 2016) (written testimony of Nancy Retzlaff).

¹⁶⁵ See Andrew Pollack, *Drug Goes From \$13.50 a Tablet to \$750, Overnight*, N. Y. Times (Sept. 20, 2015)

¹⁶⁶ See Press Release, *Turing Reduces Cost of Daraprim* (Nov. 24, 2015), *found at*, <https://www.turingpharma.com/press-releases/15/turing-reduces-cost-of-daraprimreg-pyrimethamine/> (last visited Nov. 26, 2016).

hospitals only, but that still represented a price increase of over 2,600 percent from the price of the drug when it was purchased from Impax.¹⁶⁷ The price remained unchanged for all other users. Additionally, Turing started selling the product in a 30-count bottle.¹⁶⁸

1. Gold Standard

Turing's internal documents show that the company understood Daraprim's unique value as the "gold standard" for treating toxoplasmosis. A presentation Turing shared with its investors repeatedly referenced the fact that Daraprim (in combination with sulfonamide) is "the Gold Standard of care for toxoplasmosis."¹⁶⁹ Another internal Turing analysis stated that "physicians would prefer not to have to substitute another drug . . . [and] are at a loss to think of an appropriate alternative . . ."¹⁷⁰ Turing was confident that physicians would complete prior authorization forms required by insurers for reimbursement, and go out of their way to make sure their patients had access to the drug. Further, Turing predicted that some physicians would press their patients to accept the higher cost of Daraprim in the interest of receiving the best available treatment.¹⁷¹ Under questioning by the Committee, Mr. Urrutia admitted that a drug is more valuable when it is considered to be the "gold standard" for the condition it treats.¹⁷²

Daraprim's value as the "gold standard" was not diminished by the fact that Bactrim, a sulfa-based drug, was used by a "very small subset" of physicians to treat the disease.¹⁷³ Although Bactrim is commonly used as a "maintenance" drug for patients whose acute toxoplasmosis has been brought under control, it is considered a substandard alternative to Daraprim.¹⁷⁴

2. Sole-Source

Daraprim was a sole-source drug, and Turing attempted to lock up the supply of its API, pyrimethamine, to ensure it remained so. Mr. Urrutia admitted that one of the factors Turing analyzed in valuing the Daraprim transaction was that it was a "sole-source" drug.¹⁷⁵ He also

¹⁶⁷ *Id.*

¹⁶⁸ *Id.*

¹⁶⁹ Email from Edwin Urrutia to Martin Shkreli, TUR-SCA00000620 (Jun. 17, 2015), and accompanying attachment, Project Dart, TUR-SCA00002406, at TUR-SCA00002407 (June 2015); see also, Email from Edwin Urrutia to Dan Wichman, TUR-SCA00105564 (Jun. 11, 2015), and accompanying attachment, Project Dart, TUR-SCA00105565 at TUR-SCA001105566 (Jun. 2015); Urrutia Deposition, at 156:11–157:15: (discussing sharing version of project Dart presentation with investors).

¹⁷⁰ Email from Tina Ghorban to Nancy Retzlaff, TUR-SCA00030992 (Nov. 13, 2015), and accompanying attachment, Assessing the Market Potential for Sulfadiazine and Pyrimethamine, TUR-SCA00030993, at TUR-SCA00031002 and TUR-SCA00031019 (Jun. 10, 2015).

¹⁷¹ *Id.* at TUR-SCA00031019.

¹⁷² Urrutia Deposition, at 144:10–17.

¹⁷³ Email from Tina Ghorban to Nancy Retzlaff, TUR-SCA00030992 (Nov. 13, 2015), and accompanying attachment, Assessing the Market Potential for Sulfadiazine and Pyrimethamine, TUR-SCA00030993, at TUR-SCA00031000 (Jun. 10, 2015).

¹⁷⁴ *Id.* at TUR-SCA00031000 and TUR-SCA00031013.

¹⁷⁵ Urrutia Deposition, at 202:14–20.

testified that he would have been concerned if he found out that there were multiple sources of the API available to the U.S. market.¹⁷⁶

Emails between Turing executives during the negotiations with Impax to acquire Daraprim underscore Turing's interest in acquiring sole-source drugs. In an email to Ms. Retzlaff and Ms. Tina Ghorban, dated April 29, 2015, Mr. Smith highlighted Daraprim and another drug as acquisition targets because they were "sole-source."¹⁷⁷ As Ms. Ghorban explained, she understood Mr. Smith's email to indicate that because Daraprim was both "sole-source" and the "gold standard" there was "potential for revenue" in the numbers projected.¹⁷⁸

In an email to a potential investor outlining the prospective acquisitions of Daraprim and another drug, Mr. Urrutia also stressed that both were "sole sourced and the standard of care" for the diseases they treated, and that Turing intended to place both in closed distribution.¹⁷⁹ Mr. Urrutia emphasized that Turing believed that the drugs could each generate 30 times their prior revenue and \$2 billion or more in value for the company. At the time, these two small market drugs were generating annual revenues of \$6 million and \$10 million.¹⁸⁰

At the time Turing acquired Daraprim, only two companies produced its API pyrimethamine for the U.S. market: Fukuzyu Pharmaceutical Co., Ltd., an API manufacturer located in Toyama, Japan, and IPCA Laboratories, located in India.¹⁸¹ Turing believed that an ANDA may have been filed in 2014 based on API produced by IPCA, but was confident that IPCA faced "substantial manufacturing issues that would significantly disrupt any filing" using its API.¹⁸² Turing also believed that Fukuzyu was under an exclusive supply agreement with Impax at that time.¹⁸³ In May 2015, Turing attempted to secure an exclusive deal to acquire the API from Fukuzyu for the U.S. market, but was unsuccessful.¹⁸⁴

Turing emphasized to potential investors that it expected Daraprim to remain a sole-source drug for at least a year, and possibly much longer.¹⁸⁵

¹⁷⁶ *Id.* at 203:19-23.

¹⁷⁷ Email from Michael Smith to Nancy Retzlaff and Tina Ghorban, TUR-SCA00030775 (Apr. 29, 2015).

¹⁷⁸ Transcribed Interview of Christina Ghorban, at 62:5-22 (Mar. 10, 2015) ("Ghorban Interview"). *See also id.* at 9:7-19 (Ms. Ghorban acknowledging the content of 18 U.S.C. §§ 1001 and 1505 and the fact that those statutes applied to transcribed interview).

¹⁷⁹ Email from Edwin Urrutia to Dan Wichman, TUR-SCA00007881 (May 20, 2015).

¹⁸⁰ *Id.*

¹⁸¹ *See* Email from Tina Ghorban to Nancy Retzlaff, TUR-SCA00031037, and accompany attachment, Project Dart, TUR-SCA00031038, at TUR-SCA00031043 (Jun. 2015).

¹⁸² *Id.*

¹⁸³ *Id.*

¹⁸⁴ *See generally*, Email from Michael Smith to Martin Shkreli, TUR-SCA00007901 (Jun. 1, 2015).

¹⁸⁵ *See* Email from Tina Ghorban to Nancy Retzlaff, TUR-SCA00031037, and accompany attachment, Project Dart, TUR-SCA00031038, at TUR-SCA00031046 (Jun. 2015).

3. Small Market

In the lead-up to the acquisition of Daraprim, Mr. Shkreli appears to have asked Mr. Smith to analyze the probability that a decades-old drug would face generic competition given the number of units sold and net revenues received annually.¹⁸⁶ After reviewing the competitive history of nearly 5,500 products, Mr. Smith reported back that “the most important takeaway” from his analysis was that just 10.8 percent of off-patent drugs with under \$10 million in annual sales faced generic competition within three years.¹⁸⁷ Furthermore, there was only a five percent probability that a generic competitor would enter a market where fewer than 20,000 units of a drug were sold each year.¹⁸⁸ Turing emphasized to investors that it was unlikely Daraprim would face a generic competitor soon—only 9,708 units (bottles) of Daraprim were sold in 2014 and net sales of the drug were under \$5 million.¹⁸⁹

Mr. Shkreli and Mr. Smith were not alone in their view that a small patient population was critical for remaining unchallenged after raising the price of Daraprim. Mr. Urrutia testified that Daraprim’s most attractive feature was the fact that it is served a small patient population.¹⁹⁰ He explained that small patient populations require a lot of effort and resources to serve, and this means that companies that own drugs that treat serious conditions for small populations have “pricing power.”¹⁹¹ Turing believed its planned price-hike would go unchallenged because so few people would be affected by it. As one internal document put it, “[t]he number of toxoplasmosis patients is too small to stimulate a significant lobbying effort were the cost of therapy to become an issue.”¹⁹²

4. Restricted Distribution

Daraprim is in “restricted distribution,” under which the drug cannot be obtained through normal pharmacy channels, but instead must be obtained from so-called “specialty” pharmacies. The restricted distribution arrangement was put in place previously by Impax, but used by Turing to tightly control the drug’s distribution to lock out potential competitors. As previously noted, some companies use restricted distribution due to a FDA required REMS protocol.¹⁹³ Daraprim is not subject to FDA mandated REMS.

¹⁸⁶ See Urrutia Deposition, at 153:18–155:7; Deposition of Michael Smith, at 131:14–132:5 (Apr. 3, 2016) (“Smith Deposition”).

¹⁸⁷ Email from Michael Smith to Martin Shkreli, TUR-SCA00007887, at TUR-SCA00007888 (May 23, 2015).

¹⁸⁸ *Id.*

¹⁸⁹ See Email from Edwin Urrutia to Martin Shkreli, TUR-SCA00000620 (Jun. 17, 2015), and accompanying attachment, Project Dart, TUR-SCA00002406, at TUR-SCA00002409 (June 2015).

¹⁹⁰ See Urrutia Deposition, at 204:23–205:5.

¹⁹¹ *Id.* at 85:13–20.

¹⁹² Email from Tina Ghorban to Nancy Retzlaff, TUR-SCA00030992 (Nov. 13, 2015), and accompanying attachment, Assessing the Market Potential for Sulfadiazine and Pyrimethamine, TUR-SCA00030993, at TUR-SCA00031020 (Jun. 10, 2015).

¹⁹³ See, *supra*, at 20.

Restricted distribution in this case was a deliberate part of Turing’s plan to defend its shocking price increase and subsequent increased revenue against potential competition. As Mr. Dorfman explained, the closed distribution/specialty pharmacy arrangement:

[C]an reduce, if not eliminate, the opportunity for a second generic entrant to furnish sufficient quantities of the drug to patients in order to complete the necessary bioequivalence studies required for FDA approval. In the case of Daraprim, the retention of a new specialty pharmacy distributor to carry on a closed distribution system distribution system was considered an integral part of the company’s desire to block a generic entrant for at least three years.¹⁹⁴

Internal Turing documents demonstrate the prevalence of this strategy at Turing. In a pre-transaction email in which Turing was exploring the acquisition of both Daraprim and Sulfadiazine, Mr. Smith wrote to Ms. Retzlaff and Ms. Ghorban, “[a]nother item to keep on your radar is Sulfadiazine. It is a sole-source (US only, generic ex-us) infectious disease product from Sandoz, indicated for toxoplasmosis. This would be the classic closed distribution play—we think it could do >250mm per annum.”¹⁹⁵ Despite this email, Mr. Smith later maintained that he wasn’t exactly sure what the term “classic closed distribution play” referred to and that such a “classic closed distribution play” could decrease the value of a drug to Turing.¹⁹⁶ This was directly contradicted not only by Mr. Dorfman’s recollection, but by Ms. Ghorban who stated her view that “the business development team,” which included “Michael Smith,” believed that “classic closed distribution play” refer[ed] to the concept that you could use closed distribution to make it more difficult to get referenced listed drug for bioequivalence studies.”¹⁹⁷

In another such email, Ms. Retzlaff instructed Ms. Ghorban that in anticipation of the Daraprim transaction closing in a matter of weeks: “As you’re both aware, the priority work stream is to ensure the product is moved in a closed distribution as swiftly as possible in order to minimize exposure.”¹⁹⁸ Ms. Ghorban indicated to the Committee that Ms. Retzlaff’s email may have been referencing the strategy of using closed distribution to prevent generic entry because that was “what Martin [Shkreli] and the B[usiness] D[evelopment] team had been talking about” in June of 2015.¹⁹⁹

In addition, in July 2015, Mr. Shkreli texted Mr. Smith: “Interesting point from call with Kurt. Generics are required to keep samples on hand. So closed will prohibit new guys but any ANDA filer will keep samples and expiry won’t matter cause they can retest them.”²⁰⁰

¹⁹⁴ March 2016 Hearing, at 2–3 (Written Testimony of Howard Dorfman, Esq.).

¹⁹⁵ Email from Michael Smith to Nancy Retzlaff and Tina Ghorban, TUR-SCA00030775 (Apr. 29, 2015).

¹⁹⁶ Smith Deposition, at 93:3–100:12.

¹⁹⁷ Transcribed Interview of Christina Ghorban, at 47:8–11, 47:22, and 48:4–7 (Mar. 10, 2015) (“Ghorban Interview”). *See also id.* at 9:7–19 (Ms. Ghorban acknowledging the content of 18 U.S.C. §§ 1001 and 1505 and the fact that those statutes applied to transcribed interview).

¹⁹⁸ Email from Nancy Retzlaff to Tina Ghorban, TUR-SCA00282415, at TUR-SCA00282415 (June 10, 2015).

¹⁹⁹ Ghorban Interview, at 86:2–17.

²⁰⁰ Michael Smith Text Messages, TUR-SCA00289320, at TUR-SCA00289320 (July 11, 2015).

Mr. Shkreli and other senior Turing executives touted to potential investors the ability of closed distribution to block generic entry. As Mr. Urrutia explained, “investors get excited about a specialty distribution system because it does limit the generics that are able to access your product.”²⁰¹ He added when explaining a portion of a presentation that Turing prepared to highlight its business strategy, “closed distribution can increase a product life cycle by preventing generics from potentially getting your referenced product.”²⁰²

Mr. Smith maintained his denial that Turing actually pursued this strategy at the Committee’s March 2016 Hearing. There, he characterized Turing’s investor facing material as merely “suggesting” the use of a strategy to block generic entry through restricted distribution:²⁰³ “Turing supplies Daraprim in large volumes to some institutional purchasers. We don’t have the ability to control access to the product once it goes into those channels.” This claim—that Turing really didn’t mean to implement a restricted distribution strategy because it would not be effective in restricting institutional purchasers (such as 340B hospitals), and accordingly did not do so—is contradicted by the record.

To be sure, sales to 340B institutions were not strictly part of the restricted distribution system discussed above. But they were also tightly controlled to ensure that the drugs would not fall into the hands of potential generic entrants. Mr. Shkreli instructed Turing employees to ensure that Turing bought back all existing inventory in the distribution system to ensure (in part) that Turing had complete control over every bottle of Daraprim that left its hands.²⁰⁴ Further, Turing imposed a 5 bottle per transaction limit on sales to institutional purchasers, explaining that this rule “is to ensure that the account is legit and not a generics manufacturer.”²⁰⁵ Turing tightly monitored this channel, at times refusing to ship product until they confirmed it was going to actual 340B institutions to meet immediate patient need (as opposed to going to a generic manufacturer or being stockpiled by a 340B institution).²⁰⁶ In one instance, when Turing thought its logistics vendor had shipped Daraprim to a compounding pharmacy, its employees reacted angrily and swiftly, writing:

We do not sell Daraprim to compounding pharmacies. . . . This needs to be researched immediately and we need a detailed explanation and a rapid remedy (if the situation is as

²⁰¹ Urrutia Deposition, at 77:11–14.

²⁰² Urrutia Deposition, at 211:10–13; *see also, id.* at 206:3–22 (identifying document discussed in cited passage as Email from Edwin Urrutia to Ron Tilles, TUR-SCA00174150 (Nov. 13, 2015), and *accompanying attachment*, HC Fund, TUR-SCA00174151, at TUR-SCA00174152 (undated)); *id.* at 142:10–143:22 (similar discussion regarding closed distribution).

²⁰³ *See* March 2016 Hearing, at 2 (written testimony of Michael Smith); *id.* Trans, at 129:2–7 (testimony of Michael Smith).

²⁰⁴ *See* Email from Martin Shkreli to Michael Smith, TUR-SCA00006828, at TUR-SCA00006828–29 (Aug. 11, 2015).

²⁰⁵ Email from Jon Hass to Rick DeYoung, TUR-SCA00123435, at TUR-SCA0012345 (Oct. 2, 2015); *see also*, Email from Nila Desai to John Hass and Tom Evegan, TUR-SCA00197552, at TUR-SCA00197552 (implementing policy).

²⁰⁶ *See generally, e.g.*, Email from Tina Ghorban to Nancy Retzlaff, TUR-SCA00120149 (Oct. 30, 2015); Email from Ann Parkinson to Tom Evegan et al., TUR-SCA00054581 (Oct. 29, 2015).

I suspect it is) to get those bottles returned and destroyed at your expense. I need an immediate response and action.²⁰⁷

Ms. Retzlaff reiterated this directive emphatically, writing “[w]e need to get those bottles stat.”²⁰⁸ The record speaks for itself. Turing used restricted distribution—across all purchasing channels with the intent to block generic entry.

5. Price Gouging

Turing attempted to justify the outrageous price increase for Daraprim by arguing that it would use the resulting revenues to increase research and development for an improved toxoplasmosis treatment. Yet Mr. Dorfman testified that “the price increase, as contemplated and subsequently announced, was not justified by any such actual expenditure.”²⁰⁹ He explained:

[The] pharmaceutical industry has historically worked toward measuring price increases with the expenditures that were needed to establish clinical trial programs and to be able to fund a drug basically from the lab through FDA approval. These were not expenses or costs that Turing had incurred . . . and the rationale for that kind of price increase, or any major price increase, was lacking.²¹⁰

He considered the price increase “not justifiable” and “unethical.”²¹¹

At the Committee’s March 17, 2016, Hearing, Mr. Tilles claimed that “the revenues generated by Daraprim are primarily used to fund R&D.”²¹² But the extraordinary price hike Turing imposed on Daraprim had nothing to do with the R&D necessary to develop that drug. When questioned by Chairman Collins, Mr. Tilles admitted that the Daraprim pill Turing is selling today is essentially the same pill that has been on the market since the drug was approved in 1953, over a half-century before Turing was founded.²¹³ Turing made no changes to the pill except to raise its price from \$13.50 to \$750.00 per pill—more than 5,000 percent—overnight.

To put the price increase into perspective, before Turing bought the drug, a full year’s treatment would have cost \$6,500 and after Turing acquired it, the cost rose to \$361,000.²¹⁴

²⁰⁷ Email from Nancy Retzlaff to Jon Hass et al., TUR-SCA00196428, at TUR-SCA00196428 (Nov. 13, 2015).

²⁰⁸ Email from Nancy Retzlaff to Jon Hass et al., TUR-SCA00196428, at TUR-SCA00196428 (Nov. 13, 2015). This episode was resolved when it was determined that the Daraprim recipient was not a compounding pharmacy, but was in fact a 340B institution. Email from Rick DeYoung to Nancy Retzlaff, TUR-SCA00191764, at TUR-SCA00191764–65 (Nov. 13, 2015).

²⁰⁹ Senate Special Committee on Aging Hearing, *Sudden Price Spikes In Decades Old Rx Drugs: Inside the Monopoly Business Model*, at 3 (Mar. 17, 2016) (written testimony of Howard Dorfman, Esq.).

²¹⁰ March 2016 Hearing, Trans. at 49:5–15 (testimony of Howard L. Dorfman, Esq.).

²¹¹ Deposition of Howard L. Dorman, Esq., at 73: 16–17 and 74:18–19 (Feb. 2, 2016).

²¹² March 2016 Hearing, at 2 (written testimony of Ron Tilles).

²¹³ *Id.* Trans. at 74:7–18.

²¹⁴ The exact dosage and subsequent calculations depend on the age of the patient and the stage of the disease.

Mr. Tilles attempted to justify the price increase by arguing that some patients tolerate Daraprim poorly and that future improvements needed to be made to the drug.²¹⁵ But the overwhelming majority of physicians interviewed indicated that Daraprim is a highly effective treatment for toxoplasmosis and is well-tolerated, so this is not an area where a new drug is urgently needed.²¹⁶

Mr. Smith, another Turing executive who testified at the March 2016 Hearing, claimed to “care deeply” about patients who needed Daraprim. Copies of Skype chats obtained by the Committee between Mr. Smith and other Turing employees belie this sentiment. Chairman Collins confronted Mr. Smith with the chats:

. . . Mr. Smith, you made the most outrageous statement of anyone when you stated, quote, that you “care deeply about the patients who take Daraprim.” I would like to draw your attention to Exhibit 8 and Exhibit 9.

Now, clearly, you know that Daraprim went from being modestly priced to being prohibitively expensive. And when you started to hear of access problems, according to a Skype exchange that we have, which is shown in Exhibit 8, you say, “I think some of them are fake.”

You participated in a Skype chat with two of your colleagues in which you express shock that two patients had paid cash for Daraprim. Let me read from that chat, although it is difficult to do so because of the number of expletives in it in which you are making fun of patients that are paying the full amount. “Two patients have paid cash for Daraprim. Rich [expletive deleted]. Oh, my God. Wow.”

You went on to discuss concerns that you had with the 340B . . . program. . . . [Y]ou express concern that it was cutting into Turing’s profits. So, as we can see from the slides, your colleague, Mr. Crutcher, wrote, “Time to dip out of the 340B claims [expletive deleted]. F- these guys.” Your reply, “Laugh out loud. Yes. I told her to start disputing the 340B claims.”²¹⁷

A review of Turing internal documents shows the real reason for the price increase—the desire to maximize its profits. In an email to Mr. Jim Silverman, of Opaleye Management (a Boston-based hedge fund), Mr. Shkreli estimated that Turing’s \$55 million investment in Daraprim would yield annual revenues exceeding \$200 million.²¹⁸ Other documents shared with investors predicted “[p]otential revenues of over \$500 million, and 80% EBITDA.”²¹⁹ When asked by another apparent investors for the Daraprim “projections,” Mr. Shkreli wrote:

²¹⁵ See March 2016 Hearing, Trans. at 74:7–75:5.

²¹⁶ See, e.g., Committee Staff Interview with Dr. David Kimberlin (Nov. 20, 2015); Committee Staff Interview with Dr. Jose Montoya (Dec. 3, 2015). Some of the physicians the Committee spoke to did indicate that developing a drug to kill the cysts that remained latent in the body would be useful. *Id.*

²¹⁷ March 2016 Hearing, Trans. at 71:3–72:3.

²¹⁸ See Email Martin Shkreli to Jim Silverman, TUR-SCA00007941 (Aug. 8, 2015).

²¹⁹ Email from Edwin Urrutia to Martin Shkreli, TUR-SCA00000620 (Jun. 17, 2015), and accompanying attachment, Project Dart, TUR-SCA00002406, at TUR-SCA00002422 (June 2015).

I think it will be huge. We raised the price from \$1,700 per bottle to \$75,000. Previously impax sold 10,000 bottles per annum (50% is given away, however). So 5,000 paying bottles at the new price is \$375,000,000—almost all of it is profit and I think we will get 3 years of that or more. Should be a very handsome investment for all of us. Let's all cross our fingers that the estimates are accurate.²²⁰

And in perhaps his most infamous statement, when Mr. Tilles informed Mr. Shkreli that Mission was willing to entertain an offer to acquire the rights to Daraprim, Mr. Shkreli replied: "Very good. Nice work as usual. *\$1bn here we come.*"²²¹

II. Retrophin, Inc.

A. Company Background

Retrophin became publically traded on NASDAQ in December 2012.²²² Its founder was Mr. Shkreli.²²³ On May 29, 2014, Retrophin acquired the rights²²⁴ to the drug Thiola, a drug that first went on the market in 1988. Four months later, Retrophin raised the price of Thiola from \$1.50 per tablet to \$30.00 per tablet, an increase of 1,900 percent.²²⁵

On September 30, 2014, Retrophin's Board replaced Mr. Shkreli as CEO for a variety of alleged improprieties.²²⁶ Mr. Shkreli resigned his positions at Retrophin on October 13.²²⁷ Retrophin then sued Mr. Shkreli for some \$65 million dollars in damages and raised detailed accusations that Mr. Shkreli improperly raided corporate resources to enrich himself and to pay off investors in his prior hedge fund which had lost an enormous amount of money on a bad deal.²²⁸ Mr. Shkreli countersued, asserting claims under his employment agreement.²²⁹ There is a difference between the Retrophin run by Mr. Shkreli, and the Retrophin after the departure of

²²⁰ Email from Martin Shkreli to Greg Rea, TUR-SCA00008319 (Aug. 27, 2015).

²²¹ Email from Martin Shkreli to Ron Tilles, TUR-SCA00000503, at TUR-SCA-00000503 (May 27, 2015).

²²² See Retrophin, *Retrophin Complete Reverse Merger with Desert Gateway, Inc.* (Dec. 18, 2012), found at, http://files.shareholder.com/downloads/AMDA-1PLA2O/3336041822x0x670119/5221E841-2D8C-4C10-ADE3-3B68CB00B534/RTRX_News_2012_12_18_General_Releases.pdf (last visited Nov. 30, 2016).

²²³ *Id.*

²²⁴ See Retrophin, *Retrophin Enters into U.S. License Agreement for Thiola® (Tiopronin)* (May 29, 2014), found at, <http://ir.retrophin.com/releasedetail.cfm?ReleaseID=851378> (last visited Nov. 30, 2016); Email from Courtney Bond to Martin Shkreli, SSQA_THIOL_003113 (May 22, 2014), and accompanying attachment, Retrophin Investor Presentation, SSQA_THIOL_003114, at SSQA_THIOL_003117 (May 2015).

²²⁵ See Jeremy Stahl, *That Guy Who is Price-Gouging Aids Patients Also Did it to Kids with Kidney Disease*, Slate (Sept. 22, 2015).

²²⁶ See Retrophin, *Retrophin Announces Leadership Reorganization* (Sept. 30, 2014), found at, <http://ir.retrophin.com/releasedetail.cfm?ReleaseID=873875> (last visited Nov. 30, 2016).

²²⁷ See Complaint, at ¶ 3, *Retrophin, Inc. v. Shkreli*, No. 1:15-cv-06451 (NRB) (S.D.N.Y. Aug. 17, 2015) (ECF No. 1).

²²⁸ See generally, *id.*

²²⁹ See Letter Addressed to Judge Naomi Reice Buchwald from Celia G. Barenholtz, at 4, *Retrophin, Inc. v. Shkreli*, No. 1:15-cv-06451 (NRB) (S.D.N.Y. Sept. 18, 2015) (ECF No. 13) (discussing counterclaim by Mr. Shkreli in arbitration).

Mr. Shkreli. While the current company has not reversed the price increase on Thiola, it has made considerable investments in patient assistance for Thiola and appears to have renounced Mr. Shkreli's business model.²³⁰

As mentioned previously, several individuals who worked on Retrophin's acquisition of Thiola later followed Mr. Shkreli to Turing, including Mr. Urrutia, Mr. Crutcher, Mr. Smith, and Mr. Tilles, each of whom were examined on the record as part of the Committee's investigation.

B. Thiola Background

Thiola received FDA approval in 1988 and is used to treat cystinuria.²³¹ The drug prevents the buildup of kidney stones, which if untreated can be extremely painful and require surgery or lead to life threatening renal failure. While there are other drugs that treat cystinuria (such as Valeant's Cuprimine), Thiola is considered the standard of care²³² and is the only viable treatment for many patients. It is not widely prescribed, but some individuals are on the drug for long periods of time.²³³

In May of 2014, Retrophin acquired a license for the rights to Thiola from Mission Pharmaceuticals ("Mission"). Under the license agreement, Mission still owns and makes Thiola, but all marketing rights, including setting the price, were transferred to Retrophin. Mission receives a 20 percent royalty on Thiola sales. Mission previously licensed Thiola from UT Southwestern and was selling it for many years.²³⁴

In September 2014, Retrophin raised the price of the drug from \$1.50 to \$30.00 a tablet. According to Retrophin, Mr. Shkreli wanted to increase the price by a substantially greater amount, but was blocked from doing so by senior Retrophin officials.²³⁵

C. The Acquisition of Thiola

The business model later used by Turing to acquire and reprice Daraprim appeared to have first been tried by Retrophin on Thiola. A presentation made by Retrophin to its investors contemporaneous with the Thiola acquisition explained that a key reason for the company's interest in the drug was its "strong fit with Retrophin's focus on rare and catastrophic diseases." Retrophin viewed the drug as "significantly underpriced relative to the benefit it offers" to

²³⁰ Committee Staff Interview with Stephen Aselage (Nov. 14, 2016) ("Aselage Interview").

²³¹ See Retrophin Investor Presentation, SSCA_THIOL_003114, at SSCA_THIOL_003118 (May 2015).

²³² See Martin Shkreli, Transcript of licensing call regarding Thiola, SSCA_THIOL_003158 (May, 2014).

²³³ See Dr. Benjamin J. Davies, Associate Professor of Urology at the University of Pittsburgh School of Medicine, Retrophin Assailed for "Exorbitant" Price Hike (Sept. 10, 2014), *found at*, <https://www.thestreet.com/story/12873639/1/retrophin-assailed-for-exorbitant-drug-price-hike.html> (last visited Dec. 19, 2016).

²³⁴ See Email from Jim Self to Martin Shkreli, SSCA_THIOL_024838 (May 15, 2014), and accompanying attachment, Thiola Trademark License and Product Supply Agreement, SSCA_THIOL_024839. See also Email from Courtney Bond to Martin Shkreli, SSCA_THIOL_003113 (May 22, 2014), and accompanying attachment, Retrophin Investor Presentation, SSCA_THIOL_003114, at SSCA_THIOL_003117 (May 2014).

²³⁵ Aselage Interview.

patients, and the company believed it could “grow both volume and pricing” of the drug.²³⁶ The presentation went on to describe how Thiola met most of the key elements of the business model.

1. Gold Standard

The presentation explained that Thiola was one of only two drugs approved for the treatment of cystinuria, but was the “preferred therapy due to its reduced risk of adverse events.”²³⁷

2. Sole-Source

The presentation made clear that there were no known generic competitors to Thiola at the time of the acquisition.²³⁸

3. Small Market

Data in the investor presentation showed that Thiola’s annual revenues had been under \$2 million in each of the preceding five years.²³⁹ In an email exchange, Retrophin estimated that only 300 to 400 patients were on Thiola at the time.²⁴⁰ Internal Retrophin economic models showed a patient level of 394 in 2014, rising to 408 in 2015 and remaining steady thereafter.²⁴¹ Mr. Shkreli emphasized the importance of a small market to Retrophin’s ability to raise Thiola’s price without facing competition, assuring investors that few companies “will step up and care for just a handful of patients” because “low-revenue drugs are extremely low priorities for almost all drug companies.”²⁴²

4. Restricted Distribution

Retrophin also made clear that it would place the drug into closed distribution. Retrophin was upfront about its rationale, explaining that a “[c]losed distribution system prevents generics from accessing the product for bioequivalence studies.”²⁴³ In another email exchange, Mr. Shkreli speculated that denying access to the drug to potential competitors could prevent a generic entry for years, even if legislation were passed to require companies to share access:

²³⁶ Email from Courtney Bond to Martin Shkreli, SSCA_THIOL_003113 (May 22, 2014), and accompanying attachment, Retrophin Investor Presentation, SSCA_THIOL_003114, at SSCA_THIOL_003117 (May 2014).

²³⁷ *Id.* at SSCA_THIOL_003118.

²³⁸ *Id.*

²³⁹ *Id.*

²⁴⁰ See Email from Courtney Bond to Nikhil Goel, SSCA_THIOL_000638, at SSCA_THIOL_000638 (May 22, 2014).

²⁴¹ Email from Mark Panoff to Evan Greebel et al., SSCA_THIOL_038407, and accompanying attachment, Excel Spreadsheet, SSCA_THIOL_038408 (May 15, 2014).

²⁴² Email from Martin Shkreli to Courtney Bond et al, SSCA_THIOL_003157, and accompanying attachment, Thiola Licensing Call, May, 2014. SSCA_THIOL_003158, at SSCA_THIOL_003158–59 (May 2014).

²⁴³ Email from Courtney Bond to Martin Shkreli, SSCA_THIOL_003113 (May 22, 2014), and accompanying attachment, Retrophin Investor Presentation, SSCA_THIOL_003115 at SSCA_THIOL_003121 (May 2014).

It should take a long amount of time because the Sherman act clearly states companies like Retrophin and Celgene have “no duty to deal” and the Supreme Court ratified two challenges to this in the Pac Bell and Verizon cases. So if they can get some legislative momentum and get a law signed, there will still be a ‘test case’ which has to prove this law supersedes the Sherman Act, which you may know is one of the oldest American pieces of legislation. So I think worst case we have another 5 years because once we hand over samples to a generic, they will have to spend the next 3 years getting an ANDA approved.²⁴⁴

Outside analysts also noted Retrophin management’s commitment to a closed distribution system as a means of fending off generic competition “as no product may be available to conduct bioequivalent studies.”²⁴⁵

Mr. Shkreli also emphasized the importance of closed distribution to the business model in a call with investors:

[C]losed distribution [...] allows us to control the release of our product. *We do not sell Retrophin products to generic companies.* . . . The whole model that generics rely upon is turned upside down with specialty pharmacy distribution.”²⁴⁶

5. Price Gouging

Mr. Shkreli was remarkably candid regarding his drug-pricing philosophy in the context of Thiola. In an email to one investor, he put it this way:

The drug companies are afraid. Small ones, big ones, etc. Big price increases are horrifying because most executives overestimate changes in demand. It comes mostly from pharma’s history as quasi-consumer products. . . . The next generation of pharma guys (or the smart ones) understand the inelasticity of certain products. The insurers really don’t care. They just pass it through and focus on managing care for physician payments and blockbusters. They assume someone will genericize it if it is making too much money, and they’re right.

So I don’t really think of it the same way as others. I think this deal, if we pull it off, is worth \$100m-\$200m to our company. We’ll see!

I figure this dynamic may not last forever, you need to maximize opportunities while you can.

We’d pay \$1m to acquire a drug called Thiola, which is the only treatment for a rare

²⁴⁴ Email from Martin Shkreli to Dan Wichman, SSCA_THIOL_038413 (Sept. 22, 2014).

²⁴⁵ Email from Christopher Cline to Stephen Aselage et al., SSCA_THIOL_041104 (Apr. 2, 2015), and accompanying attachment, Leerlink RTRX Initiation, SSCA_THIOL_041106, at SSCA_THIOL-041118 (Apr. 2, 2015).

²⁴⁶ Email from Martin Shkreli to Courtney Bond et al., SSCA_THIOL_003157, and accompanying attachment, Thiola Licensing Call, May, 2014. SSCA_THIOL_003158, at SSCA_THIOL_003160 (emphasis added).

disease called cystinuria (contrast with RPTP cystinosis—totally different).

The drug does \$1.2m in sales. It is woefully underpriced and would not stop selling at orphan prices. With new pricing we estimate sales of \$20 to \$40 million. Almost 95% EBITDA margins at those prices. Would be an annuity for some time.

A \$100m present for you this morning.²⁴⁷

Even Mr. Shkreli's bold estimate of a "\$100 million present" to investors may have underestimated his view of Thiola's potential value. An economic model of the Thiola deal he created shows revenues for the drug exploding from just \$1.8 million in 2013—the last full year under Mission—\$13.1 million in 2014, \$41.5 million in 2015, and \$48.9 million in 2016. After subtracting the cost of goods sold, the royalty to Mission, operating expenses and taxes, Mr. Shkreli estimated that net income attributable to the drug would rise from \$867,000 in 2013 to \$5.3 million in 2014, \$20.5 million in 2015, and \$25.6 million in 2016, and would climb to as high as \$43.6 million in 2028. In net present value terms, the model shows that Mr. Shkreli estimated that Thiola would add \$291.7 million to Retrophin's valuation, equating to \$11.67 for each of the company's 25 million shares.²⁴⁸

Ultimately, the substantial price increase eventually taken on Thiola was not the price increase Mr. Shkreli wanted. He wanted to take a price increase of some four times that amount, but was stopped by others at Retrophin who considered such a large increase unwise or unconscionable.²⁴⁹

III. Valeant Pharmaceuticals International, Inc.

The Committee's investigation of Valeant covered four different drugs, two of which are consumer drugs, and two of which are used primarily in the hospital setting.²⁵⁰

A. Company Background

Headquartered in Canada, Valeant is the largest of the four companies investigated by the Committee. It markets brand name drugs, branded generics, over-the-counter products, and medical devices in more than 100 countries, including developed and emerging markets, with a focus on eye health, dermatology, and neurology therapeutic classes.²⁵¹ The company has

²⁴⁷ Email from Martin Shkreli to Dan Wichman, SSCA_THIOL_037832, at SSCA_THIOLA_037833 (May 3, 2014).

²⁴⁸ See Email from Mark Panoff to Evan Greebel et al., SSCA_THIOL_038407 (June 24, 2014), and accompanying attachment, Excel Spreadsheet, SSCA_THIOL_038408 (May 15, 2014).

²⁴⁹ Aselage Interview.

²⁵⁰ During the investigation, the then-CEO of Valeant, Mr. Pearson did not appear for a deposition, defying a Senate subpoena compelling him to appear (although he appeared at a later date). While Mr. Pearson was not cited for criminal contempt of Congress (he later purged his contempt), his conduct stands condemned.

²⁵¹ See *Impact on the U.S. Tax Code on the Market for Corporate Control and Jobs: Hearing Before the S. Permanent Subcomm. on Investigations of the S. Comm. on Homeland Sec. & Gov't Affairs*, S. Hrg. 114–88, at 93 (July 30, 2015).

existed under its current name since 2003 and, in that time, it has expanded its operations to six continents and acquired multiple U.S. companies worth more than \$30 billion.²⁵² Valeant reported that it employed approximately 16,800 employees worldwide, and generated revenue of \$8.26 billion in 2014 with operating income of \$2.04 billion.²⁵³ Valeant's current CEO is Joseph Papa who succeeded Mr. Pearson, who served as CEO from 2008 to April 2016 and Chairman of the Board from March 2011 to January 2016.

Valeant was originally a U.S. corporation based in California. In 2010, it merged with Canada's largest publicly traded drug manufacturer, Biovail Corporation.²⁵⁴ Both companies were approximately the same size at the time.²⁵⁵ The new Valeant moved its corporate headquarters to Ontario, Canada, and then relocated to Quebec in 2012.²⁵⁶

At the time of merger, then-CEO Mr. Pearson touted the tax benefits of the deal, which was a classic corporate inversion.²⁵⁷ The Senate Permanent Subcommittee on Investigations has found that since the merger, Valeant has experienced a single-digit effective tax rate on its worldwide income.²⁵⁸

The Committee's investigation focused on four drugs marketed by Valeant where it is apparent that the company followed a more sophisticated version of the business model outlined earlier to generate enormous profits on decades old off-patent drugs.²⁵⁹ Indeed, Valeant appears to have been an early adopter of the basic structure of the business model after it discovered that two drugs it had acquired in 2010 from Aton Pharmaceuticals ("Aton"), Cuprimine and Syprine (among others), could generate substantial revenues for years before competitors could enter the market.

In addition to Mr. Pearson and Mr. Papa, key players at Valeant include Mr. Robert Rosiello, CFO from August 2015 to August 2016; Mr. Howard Bradley Schiller, Interim CEO from January 6, 2016, to February 28, 2016, CFO of Valeant from December 2011 through June 2015, and Board Director from September 2012 to June 2016; and Mr. Andrew Davis, Manager of Business Development from April 2012 to March 2013, Director of Business Development from March 2013 to September 2013, and Senior Vice President of Business Development since September 2013.

²⁵² *Id.* at 40.

²⁵³ *Valeant Pharmaceuticals, Inc., 10-K*, at 8 and 28 (Feb. 25, 2015).

²⁵⁴ *See Impact on the U.S. Tax Code on the Market for Corporate Control and Jobs: Hearing Before the S. Permanent Subcomm. on Investigations of the S. Comm. on Homeland Sec. & Gov't Affairs*, S. Hrg. 114-88, at 94 (July 30, 2015).

²⁵⁵ *Id.*

²⁵⁶ *Id.*

²⁵⁷ *Id.*

²⁵⁸ *Id.* at 95.

²⁵⁹ Mr. Pearson was removed as CEO on April 21, 2016, and was formally replaced by Mr. Papa on May 3, 2016. Under Mr. Papa, Valeant states it has repudiated its strategy of acquiring decades old drugs with a business plan which called for massive price increases, but has not lowered the list price of the drugs on which it took enormous price increases. Committee Staff Interview with Joseph Papa (Aug. 30, 2016) ("Papa Interview").

B. Cuprimine and Syprine

1. Background

Cuprimine and Syprine are predominantly used to treat Wilson disease, a rare condition (about 30,000 cases worldwide) characterized by the body's inability to process copper.²⁶⁰

Dr. Frederick Askari, M.D., Director of the Wilson Disease Center of Excellence at the University of Michigan Health System, described the pathology of the disease and its grave consequences, as well as various treatment options, at the Committee's April 27, 2016, hearing. Copper (like other essential trace elements²⁶¹) is necessary in small amounts for human life, and is found in trace amounts in a variety of items humans consume.²⁶² Normally, copper levels are regulated through a natural process by which the liver removes excess copper from the body. In the case of an individual with Wilson disease, due to a genetic defect, the liver retains excess copper, and ultimately releases it into the bloodstream, where copper accumulates to potentially toxic levels.²⁶³ The consequences of the accumulation may be felt immediately or may be delayed as the body "fills up" relatively "safe" portions of the body with excess copper.²⁶⁴ Left untreated, Wilson disease can lead to liver failure, brain damage, and death.²⁶⁵ Dr. Askari testified that ceasing treatment can lead to severe consequences in a matter of weeks and that access to drugs to treat Wilson disease are truly a matter of life and death.²⁶⁶

According to Dr. Askari, treatment options for Wilson disease use "two types of action: (1) Chelating agents that prompt the organs to release copper into the bloodstream to be filtered by the kidneys and eliminated through urine; and (2) Zinc-based therapies which prevent the body from absorbing the copper."²⁶⁷ The standard of care is to use a chelating agent at least initially to remove excess copper, possibly switching to zinc after copper levels have stabilized.²⁶⁸ Two chelating agents are available:

- **Cuprimine (penicillamine)** has been used to treat Wilson disease since 1956 and was approved by the FDA in 1965.²⁶⁹ Cuprimine treats the disease by prompting "the organs to release copper into the bloodstream to be filtered by the kidneys and eliminated through the urine."²⁷⁰ Cuprimine was the medicine of choice for treating Wilson disease for much of

²⁶⁰ National Institutes of Health, National Human Genome Research Institute, *Learning About Wilson Disease*, (Dec. 8, 2010), *found at*, <https://www.genome.gov/27532725/learning-about-wilson-disease/> (last visited Dec. 8, 2016).

²⁶¹ See W. Mertz, The essential Trace Elements, *Science* (Sept. 18, 1981), [213(4514):1332-8].

²⁶² See April 2016 Hearing, Trans. at. 20:12–14 (testimony of Frederick K. Askari, M.D.).

²⁶³ *Id.* at. 20:15–18.

²⁶⁴ Committee Staff Interview with Dr. Frederick K. Askari (Apr. 26, 2016) ("Askari Interview").

²⁶⁵ See April 2016 Hearing, at 1 (written testimony of Frederick K. Askari, M.D.).

²⁶⁶ *Id.*

²⁶⁷ *Id.*

²⁶⁸ *Id.*

²⁶⁹ See April 2016 Hearing, at 1–2 (Apr. 27, 2016) (written testimony of Frederick K. Askari, M.D.); FDA File N019853.

²⁷⁰ April 2016 Hearing, at 1–2 (Apr. 27, 2016) (written testimony of Frederick K. Askari, M.D.).

the past fifty years and many patients with the disease take Cuprimine throughout their lives. While it continues to work for most patients, it is no longer the gold standard for every patient because approximately one third of patients experience adverse side effects.²⁷¹ Conversion to far less costly zinc treatment is a viable option for some patients.²⁷² That said, some physicians are reluctant to change patients who have been stable on Cuprimine for decades to another drug as every patient reacts differently to side effects.²⁷³ Cuprimine is also approved to treat cystinuria, and rheumatoid arthritis.²⁷⁴ Depen, sold by Meda Pharmaceuticals, is also a branded penicillamine, but it is not AB substitutable for Cuprimine, meaning that the two drugs interact differently with the body and accordingly a pharmacist cannot substitute Depen for Cuprimine.²⁷⁵ There are no generic versions of either drug.

- **Syprine (trientine)** was developed in 1969 as an alternative to Cuprimine for Wilson disease and received FDA approval in 1985. Syprine is now generally considered the gold standard for treating Wilson’s disease and physicians are increasingly starting therapy with Syprine since it generally has fewer side effects.²⁷⁶ There is no other version of trientine sold in the United States.²⁷⁷

2. The Acquisition of Cuprimine and Syprine

In May 2010, Valeant acquired both Cuprimine and Syprine as part of its acquisition of Aton for \$318 million with the intention to generate high-margin revenue streams. According to a presentation made to the Valeant Board, Aton was “a specialty pharmaceutical company with a focus on ophthalmology and orphan indications.”²⁷⁸ A slide in that presentation, labeled “Strategic Rationale for Transactions,” states as to Cuprimine and Syprine: “Orphan designated drugs provide stable revenues from niche indications with limited competitors.”²⁷⁹ The presentation also states “[a]ggressive price increases have driven growth despite flat to shrinking

²⁷¹ *Id.*

²⁷² See Michael J. Schilsky et al., *Costly Choices for Treating Wilson’s Disease*, *Hepatology* Vol. 61, No. 4, at 1107 (2015).

²⁷³ Askari Interview.

²⁷⁴ Cuprimine Label (Rev. 8/2012).

²⁷⁵ See Valeant Responses to Senate Special Committee on Aging (Sept. 13, 2016).

²⁷⁶ See April 2016 Hearing, at 2 (Apr. 27, 2016) (written testimony of Frederick K. Askari, M.D.).

²⁷⁷ In addition, Wilson disease patients whose copper levels have been successfully reduced can sometimes be moved to a zinc-based drug for maintenance therapy and monitored for copper buildup. See April 2016 Hearing, at 1 (written testimony of Frederick K. Askari, M.D.).

²⁷⁸ Electronic Meeting Invitation from J. Michael Pearson to Valeant Board of Directors, VRX_SCA_00284882 (Apr. 15, 2010), and accompanying attachment, Project Atom, Presentation to Board of Directors, VRX_SCA_00284883, at VRX_SCA_00284884 (Apr. 15, 2010). Mr. Pearson, testified that he was ultimately responsible for the content of any PowerPoint Presentation placed before the Board of Directors. See Pearson Deposition, at 27:19–30:21. Pearson also indicated that “in general” “he reviewed documents that went to the Board,” and would edit those documents if he deemed it necessary. *Id.* at 27:7–18.

²⁷⁹ Electronic Meeting Invitation from J. Michael Pearson to Valeant Board of Directors, VRX_SCA_00284882 (Apr. 15, 2010), and accompanying attachment, Project Atom, Presentation to Board of Directors, VRX_SCA_00284883, at VRX_SCA_00284891 (Apr. 15, 2010). Pearson testified that although he focused on other benefits of the transaction, that this bullet point did represent a key strategic rationale for the transaction. See Pearson Deposition, at 47:13–48:14.

TRxs.”²⁸⁰ Later, the presentation highlighted another reason *why* these two drugs had such powerful pricing potential—neither faced generic competition and API sourcing was a barrier for a potential generic competitor.²⁸¹

Another Board presentation indicated that the “[a]cquisition would be strategic for the US business . . . —[p]rovide stable revenues from orphan products.”²⁸² A detailed accompanying analysis indicated that Valeant expected high gross margins on Cuprimine and Syprine.²⁸³

3. Sale of Cuprimine and Syprine to Retrophin Contemplated

In August 2012, Valeant received an unsolicited offer from Retrophin for the sale of Cuprimine and Syprine.²⁸⁴ Mr. Davis recalled that he personally dealt with Mr. Shkreli and his attorneys in processing this offer.²⁸⁵ No witness or document revealed the specifics of the offer, but Mr. Davis testified that it was formulated with an upfront payment and then payments over time.²⁸⁶

Apparently in response to this offer, Valeant prepared an analysis of Cuprimine and Syprine that was sent to Mr. Pearson, Mr. Schiller and other senior company officials.²⁸⁷ The analysis considered the drugs’ impact on total revenue where revenue from Cuprimine was stabilized through price increases to offset a decline in sales, while revenues from Syprine rose due to price increases on sales volume that remained flat.²⁸⁸

Mr. Davis testified that he was instructed to pursue the transaction by management because they felt it made sense economically.²⁸⁹ In his deposition, Mr. Davis testified that the transaction was signed, but then fell through when Retrophin failed to make the required initial immediate payment.²⁹⁰

²⁸⁰ Electronic Meeting Invitation from J. Michael Pearson to Valeant Board of Directors, VRX_SCA_00284882 (Apr. 15, 2010), and accompanying attachment, Project Atom, Presentation to Board of Directors, VRX_SCA_00284883, at VRX_SCA_00284889, (Apr. 15, 2010). It also cautioned: “Pricing backlash—recent price increases could impact volume sales or increase potential for generics.” *Id.* VRX_SCA_00284900

²⁸¹ *Id.* at VRX_SCA_00284887, VRX_SCA_00284908 (Apr. 15, 2010).

²⁸² See Email from Warren Lei to J. Michael Pearson, et al., VRX_SCA_00289745, and accompanying attachment, Project Atom Update, VRX_SCA_00289830, at VRX_SCA_00289831 (Apr. 27, 2010).

²⁸³ See Email from Warren Lei to J. Michael Pearson, et al., VRX_SCA_00289745, and accompanying attachment, Aton Acquisition Model, VRX_SCA_00289746 (Apr. 27, 2010). Prior to the close of the acquisition in May 2010, Valeant undertook an analysis of substantial price increases (Cuprimine 72 percent and Syprine 105 percent). See Email from Ryan Weldon to J. Michael Pearson, VRX_SCA_00045813 (May 3, 2010). To be sure, Valeant did not implement an immediate increase at this time. It did, however, take a meaningful price increase in June. See, *infra*, at 52.

²⁸⁴ See Deposition of Andrew Davis, at 33:14–34:2, 35:9–14 (Apr. 11, 2016) (“Davis Deposition”).

²⁸⁵ *Id.* at 34:12–22.

²⁸⁶ *Id.* at 35:9–12.

²⁸⁷ See Email from Ryan Weldon to J. Michael Pearson, et al., VRX_SCA_00076615 (Aug. 17, 2012) and accompanying attachment, Cuprimine and Syprine, VRX_SCA_00076616 (undated).

²⁸⁸ See Email from Andrew Davis to Ryan Weldon, VRX_SCA_00076589, at VRX_SCA_00076589 (Aug. 17, 2012).

²⁸⁹ See Davis Deposition, at 35:15–22.

²⁹⁰ *Id.* at 35:23–37:2.

4. “Orphan Drug Strategy”

The Committee uncovered that, beginning in late 2012, Valeant faced a situation in which its Neurology and Others business unit was failing to meet some of its budgetary targets (despite steady price increases in Cuprimine and Syprine).²⁹¹ In addition to potentially having impacted the Company’s publicly reported finances, this had the potential to result in a substantial reduction in compensation for unit executives, as the company closely tied compensation to unit performance.²⁹² Valeant’s solution was to adopt the Orphan Drug Pricing Strategy in order to meet aggressive performance goals and generate extraordinary public numbers to trumpet to Wall Street.

A five-year strategic plan reviewed by Valeant’s Board on August 31, 2012, projected that the base business of the Neurology and Other unit would erode over the planning horizon, from \$717 million to \$570 million annually—a compound rate of decline of 4.5 percent per

²⁹¹ Valeant divided its operations into a number of business units. All four drugs investigated by the Committee (Cuprimine, Syprine, Isuprel, and Nitropress) were located in the “Neurology and Other” business unit. The Neurology and Other unit was formed to hold a “hodgepodge” of products that did not fall within the other Valeant business units, such as “ophthalmology” or “dermatology.” See Pearson Deposition, at 80:12–82:19. Neurology and Others, as well as most Valeant units, reported to the Executive Vice President for the Company Group during the time relevant to the investigation.

²⁹² Mr. Pearson, in response to Committee questioning, testified that executive compensation within a Valeant business unit was linked to its performance relative to a “budget” which was established and approved by the Board prior to each fiscal year. See Pearson Deposition, at 128:3–14, 130:17–20.

Q: Okay. And to be clear, when you said you paid—get paid off the budget, I take that to mean that your bonus is linked to how your unit performs relative to your budget, in broad terms?

A: A piece of your bonus.

Q: Okay. How much?

A: So our bonus is—for the—for the business unit managers, about 75 percent is—65 to 75 percent is based on financial performance of that unit, approximately 65 percent; 10 percent is on the entire company; and 25 percent is on strategic initiatives.

Q: Okay. And what’s the order of magnitude of bonuses versus salary?

A: Twenty-five to, for the very large units, 50 percent . . . of your salary would be your target bonus.

Id. at 132:17–133:13.

This bonus regime applied across all levels of management, from the managers of individual business units to Mr. Pearson. See Deposition of Howard Bradley Schiller, at 45:8–51:3 (Apr. 6, 2016) (explaining intricacies of Valeant’s bonus scheme) (“Schiller Deposition”). Given that compensation of Valeant executives ranged from a six figure salary to a seven figure salary a year, the bonuses implicated by this regime were substantial. See Letter from Robert K. Kelner, Esq. to Samuel E. Dewey, Esq. (Apr. 26, 2016) and accompanying chart [UNDER SEAL]. See also, VRX_SCA_00595719–VRX_SCA_00595757 for detailed compensation analytics (undated) [UNDER SEAL]. And this regime may have been strictly enforced. The Controller, Tanya Carro, drafted a suggested cover email for transmitting 2015 Budget targets to corporate sectors which read. “We would hope that you would exceed your targets, but it is imperative that we not fall short.” Email from Tanya Carro to J. Michael Pearson and Howard Schiller, VRX_SCA_00389324, at VRX_SCA_00389324 (Aug. 15, 2014). *But see*, Pearson Deposition at, 216:16–217:22 (stating language in the foregoing email was not typical in his view, and noting he would not have “typically” used such language).

year.²⁹³ Even so, the plan targeted total revenues for the business unit to *grow* to \$1 billion as it sought out “life cycle management opportunities for older products,” and pursued “M&A opportunities to add new mature products to manage for cash.”²⁹⁴

Leadership in this business unit was extremely concerned about a variety of obstacles to meeting this ambitious goal for growth.²⁹⁵ Three factors appear to have led to the Valeant Orphan Drug Strategy: First, there was “[n]othing more to scrub in P&L,” *i.e.*, growth via cost savings was untenable²⁹⁶; second, “[p]ricing upsides near exhaustion;”²⁹⁷ and third, was “[v]ictims of past success’—Investor/analyst expectations exceed our guidance.”²⁹⁸

Later that year, Board members, including Mr. Schiller and Mr. Pearson, reviewed the 2013 Budget which would establish compensation metrics for the entire company.²⁹⁹ This budget projected Neurology and Other as Valeant’s most profitable U.S. unit by net revenue, and noted further price increases as a major opportunity for growth.³⁰⁰

Faced with the need to drive profits both company wide and specifically within Neurology and Other, Valeant worked quickly to develop the Orphan Drug Pricing Plan.³⁰¹ On

²⁹³ See Email from Laura Gaibor to Valeant Board, VRX_SCA_00292711 (Aug. 31, 2012), *and accompanying attachments*, Meeting Objectives and Agenda Review, Strategic Planning Offsite VRX_SCA_00292712, and US Neurology / Other Strategic Plan 2012-2017, VRX_SCA_00292739, at VRX_SCA_00292740 (June 18–20, 2012).; Pearson Deposition, at 89:16–23.

²⁹⁴ Email from Laura Gaibor to Valeant Board, VRX_SCA_00292711 (Aug. 31, 2012), *and accompanying attachments*, Meeting Objectives and Agenda Review, Strategic Planning Offsite, VRX_SCA_00292712, at VRX_SCA_00292725 (June 18–20, 2012), Neurology and Other Strategic Plan 2012–2017, Strategic Planning Offsite, VRX_SCA_00292739, at VRX_SCA_00292741, VRX_SCA_00292740, and VRX_SCA_00292751 (June 18–20); *see also*, Pearson Deposition, 91:10–19. Pearson testified that Valeant’s internal documents tend to use “revenue” as shorthand for “net revenue.” Pearson Deposition, at 79:16–80:7.

²⁹⁵ See Email from Laura Gaibor to Valeant Board, VRX_SCA_00292711 (Aug. 31, 2012), *and accompanying attachments*, Meeting Objectives and Agenda Review, Strategic Planning Offsite VRX_SCA_00292712, and US Neurology / Other Strategic Plan 2012-2017, VRX_SCA_00292739, at VRX_SCA_00292751 (June 18–20, 2012).

²⁹⁶ *Id.*

²⁹⁷ *Id.*

²⁹⁸ *Id.*

²⁹⁹ See Email from Craig Olson to J. Michael Pearson, Howard Schiller et al., VRX_SCA_00293063 (Nov. 11, 2012), *accompanying attachment*, 2013 Budget: Board of Directors Discussion, VRX_SCA_00293064 (Nov. 13, 2012).

³⁰⁰ See Email from Craig Olson to J. Michael Pearson, Howard Schiller et al., VRX_SCA_00293063 (Nov. 11, 2012), *accompanying attachment*, 2013 Budget: Board of Directors Discussion, VRX_SCA_00293064, at VRX_SCA_00293070, VRX_SCA_00293077. (Nov. 13, 2012).

³⁰¹ On January 15, 2013, Davis emailed Mr. Lei Warren (Valeant Business Development), and Mr. Jeff Strauss (Valeant Business Development) with the subject “Orphan Pricing” and asked “[w]anted to check if you have anything showing orphan drug price comps (for when making price increase argument).” Email from Warren Lei to Jeff Strauss and Andrew Davis, VRX_SCA_00076625, at VRX_SCA_00076625 (Jan. 25, 2013). In response, Mr. Lei circulated a 2011 spreadsheet that detailed how Valeant’s orphan drugs were priced much lower than other orphan drugs (without distinction as to innovator status). Excel Spreadsheet, VRX_SCA_00076627 (undated). Mr. Strauss noted: “we appear to be one of the lower cost orphan options. The conclusion could be that we could increase price significantly and still be under the radar of high cost orphan drugs.” Mr. Lei then wrote: “[m]any of the more expensive orphans are biologics. Since we’re small molecule—we have less of an argument on cost than others. Also—since we’ve taken price on others, didn’t want to take it all at once in a single year and get any potential backlash.” Email from Jeff Strauss to Lei Warren and Andrew Davis, VRX_SCA_00076712, at

April 20, 2013, Mr. Hemanth J. Varghese (“Mr. Varghese”), the then-head of Neurology and Other unit, presented the May 2013 Forecast to Mr. Pearson and Mr. Schiller.³⁰² The Executive Summary is shown in **Figure 2**.

Figure 2. Valeant’s Executive Summary



Executive Summary

- **Q1 final results**
 - Net Sales: (\$2.1M) vs LE2, \$4.4M vs Plan
 - EBITA: \$4.3M vs LE2, \$12.5m vs Plan
- **LE5 Overview**
 - Q2 Net Sales: (\$6.3M) vs LE2, \$1.1M vs Plan
 - Q2 EBITA: (\$2.0) vs LE2, \$7.9 vs Plan
 - FY Net Sales: (\$41.7M) vs LE2, (\$5.2M) vs Plan
 - FY EBITA: (\$19.9M) vs LE2, \$24.1M vs Plan
- **Key Downside Drivers: (\$47.7M) of the (\$41.7M) from LE**
 - **Partner Products:** FY (\$24.8M) vs LE2, (10.2M) vs Plan (*Slides 27-39*)
 - **WBXL:** FY (\$9.9) vs LE2, (\$13.3) vs Plan (*Slides 45-49*)
 - Q4 Medco buy >50k units (~\$10M) Q1 Medco buy 10k units (~\$3.2M). Not properly accounted for in 2013 Plan
 - Revised Forecast does not account for any future buy-ins
 - **Fenofibrate:** FY (\$13.2M) vs LE2, (\$36.8) vs Plan (*41-44*)
 - Teva request for rebids blocked conversion opportunity as they defended down to smallest accounts
 - Some success with small indirect accounts (~2%) but insufficient opportunity to fully compensate
- **Price Actions**
 - Jan 2013 revised pricing schedule upward across all products & accelerated rest of year to March for majority of portfolio
 - Exploring where additional pricing actions feasible for Q2-Q4 to compensate, including exploring orphan drug opportunity



As Mr. Pearson admitted when deposed, this slide captures the unit’s plan of making up for declining revenue by implementing major price increases.³⁰⁴ The detailed portion of the slides revealed that a 400 percent pricing increase was contemplated for Syprine, with a 100 percent increase for Cuprimine.³⁰⁵ Together with “patient advocacy/support investment,” and

VRX_SCA_00076712 (Jan. 28, 2013). Mr. Davis testified he did not recall these exchanges. Davis Deposition at 38:9–10.

³⁰² See Email from Hemanth Varghese to J. Michael Pearson and Howard Schiller, VRX_SCA_00039561 (Apr. 20, 2013), and accompanying attachment, Neurology/Other Forecast Five, VRX_SCA_00039562 (Apr. 22, 2013).

³⁰³ See Email from Hemanth Varghese to J. Michael Pearson and Howard Schiller, VRX_SCA_00039561 (Apr. 20, 2013), and accompanying attachment, Neurology/Other Forecast Five, VRX_SCA_00039562, at VRX_SCA_00039564 (Apr. 22, 2013). The “plan” referred to in this presentation is the all-important budget number. Pearson Deposition at 127:20–128:14. The forecasts are mid-year updated performance projections. *Id.* The numbers in parenthesis are negative.

³⁰⁴ See Pearson Deposition, at 130:12–17. Pearson testified that although Neurology and Other was rarely behind budget, they did at times use price increases in order to meet their budgets. *Id.* at 131:22–132:16.

³⁰⁵ See Email from Hemanth Varghese to J. Michael Pearson and Howard Schiller, VRX_SCA_00039561 (Apr. 20, 2013), and accompanying attachment, Neurology/Other Forecast Five, VRX_SCA_00039562, at VRX_SCA_00039584 (Apr. 22, 2013).

price hikes for Demser (another drug it controlled), Valeant projected incremental revenue increases of \$43.6 million.³⁰⁶

Five days later, Mr. Laizer Kornwasser, the then-head of the Company Group, reviewed an orphan pricing model designed to “get to the projected number this year that Mike [Pearson] had in his head.”³⁰⁷ This model proposed targeting a series of price hikes cumulating to 500 percent increase for Syprine and 100 percent for Cuprimine in the second half of 2013.³⁰⁸ Similar models were circulated to the most senior levels of the company in the ensuing days.³⁰⁹

In June, senior Valeant Executives reviewed drafts of a “program summary” for the “Orphan Disease Product Launch Plan.”³¹⁰ The plan was designed to implement huge price increases to drive profitability.³¹¹

In the ensuing months Valeant implemented price increases on both drugs that were *greater* than the substantial increase contemplated by the pricing model and strategy document. Mr. Pearson testified that he was generally aware of these price increases and felt they were justified because Cuprimine and Syprine were “mispriced” when compared to “what orphan products are selling for.”³¹² The price history of Syprine and Valeant are reflected in **Table 2**.

Table 2. Pricing Histories of Cuprimine and Syprine

³⁰⁶ *Id.* at VRX_SCA_00039586.

³⁰⁷ Email from Jeff Strauss to Laizer Kornwasser VRX_SCA_00077863 (Apr. 25, 2013), *and accompanying attachment*, Excel Spreadsheet, VRX_SCA_00077864 (undated).

³⁰⁸ See Email from Jeff Strauss to Laizer Kornwasser VRX_SCA_00077863 (Apr. 25, 2013), *and accompanying attachment*, Excel Spreadsheet, VRX_SCA_00077864 (undated).

³⁰⁹ See, e.g., Email from Jeff Strauss to Howard Schiller et al., VRX_SCA_00077285 (Apr. 29, 2013), *accompanying attachment*, Excel Spreadsheet, VRX_SCA_00077286 (undated); Email from Jonathan Ercole to Andrew Davis, VRX_SCA_00015895, *accompanying attachments*, Excel Spreadsheet, VRX_SCA_00015896 (undated), Excel Spreadsheet, VRX_SCA_00015992 (undated).

³¹⁰ Email from Hemant Varghese to Laizer Kornwasser, VRX_SCA_00038777 (June 27, 2013), *and accompanying attachment*, Orphan Disease Product Launch Plan, VRX_SCA_0038778 (undated); Orphan Disease Produce Launch Plan, VRX_SCA_00055326 (undated).

³¹¹ *Id.* at VRX_SCA_00055342, VRX_SCA_00055349–50. Mr. Pearson had no recollection of this document which was found on his computer hard drive. The Committee has no reason to doubt the veracity of Mr. Pearson’s representation. See Pearson Deposition at 139:18–142:5. See, e.g., Email from Howard Schiller to Amy Hancock, VRX_SCA_00105123 (Dec. 4, 2014), *and accompanying attachment*, Neuro Pricing, VRX_SCA_00105124, at VRX_SCA_00105125 (Dec. 2, 2014) (noting historical pricing variation due to “Orphan strategy launched mid 2013”).

³¹² Pearson Deposition, at 185:10–186:4.

Cuprimine					Syprine				
Date	Old Price	New Price	% Increase	Cummulative % Increase	Date	Old Price	New Price	% Increase	Cummulative % Increase
5/26/2010	\$ 444.89	\$ 444.89	0.0%		6/3/2010	\$ 652.05	\$ 782.46	20.0%	
6/3/2010	\$ 444.89	\$ 507.17	14.0%	14.0%	6/4/2010	\$ 652.05	\$ 782.46	20.0%	20.0%
6/4/2010	\$ 444.89	\$ 507.17	14.0%	14.0%	10/7/2010	\$ 782.46	\$ 821.58	5.0%	26.0%
8/2/2010	\$ 507.17	\$ 542.67	7.0%	22.0%	3/12/2011	\$ 821.58	\$ 985.90	20.0%	51.2%
3/12/2011	\$ 542.67	\$ 596.94	10.0%	34.2%	8/27/2011	\$ 985.90	\$ 1,035.20	5.0%	58.8%
8/27/2011	\$ 596.94	\$ 614.85	3.0%	38.2%	2/8/2012	\$ 1,035.20	\$ 1,128.37	9.0%	73.0%
2/29/2012	\$ 614.85	\$ 670.19	9.0%	50.6%	8/1/2012	\$ 1,128.37	\$ 1,229.92	9.0%	88.6%
8/23/2012	\$ 670.19	\$ 730.51	9.0%	64.2%	10/25/2012	\$ 1,229.92	\$ 1,291.42	5.0%	98.1%
2/1/2013	\$ 730.51	\$ 887.57	21.5%	99.5%	2/1/2013	\$ 1,291.42	\$ 1,394.73	8.0%	113.9%
4/1/2013	\$ 887.57	\$ 985.20	11.0%	121.4%	4/1/2013	\$ 1,394.73	\$ 1,548.15	11.0%	137.4%
7/12/2013	\$ 985.20	\$ 1,231.50	25.0%	176.8%	7/12/2013	\$ 1,548.15	\$ 3,049.86	97.0%	367.7%
8/9/2013	\$1,231.50	\$ 1,970.40	60.0%	342.9%	8/2/2013	\$ 3,049.86	\$ 5,703.23	87.0%	774.7%
11/1/2013	\$1,970.40	\$ 2,364.48	20.0%	431.5%	8/30/2013	\$ 5,703.23	\$10,550.97	85.0%	1518.1%
2/28/2014	\$2,364.48	\$ 2,955.60	25.0%	564.3%	2/28/2014	\$10,550.97	\$13,188.71	25.0%	1922.7%
5/30/2014	\$2,955.60	\$ 3,694.50	25.0%	730.4%	7/18/2014	\$13,188.71	\$19,783.07	50.0%	2934.0%
7/18/2014	\$3,694.50	\$ 5,541.75	50.0%	1145.6%	2/12/2015	\$19,783.07	\$19,783.07	0.0%	2934.0%
11/21/2014	\$5,541.75	\$ 6,090.38	9.9%	1269.0%	7/2/2015	\$19,783.07	\$21,266.80	7.5%	3161.5%
7/2/2015	\$6,090.38	\$ 6,547.16	7.5%	1371.6%					
7/31/2015	\$6,547.16	\$26,188.64	300.0%	5786.5%					

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Subsequent to the more than 200 percent cumulative increase (FY) on Cuprimine in July and August 2013 and the more than 800 percent cumulative increase (FY) on Syprine in the same time period, the Neurology and Other unit presented forecasts to Mr. Pearson, Mr. Schiller, Mr. Kornwasser and other senior management indicating that they were now on track to surpass their budget projection³¹⁴ (and presumably earn the attendant bonuses for doing so).³¹⁵ This success was due in no small part to the incredible profits flowing from Cuprimine and Syprine.³¹⁶ Further price increases were planned for both drugs later in the year.³¹⁷

The 2014 budget³¹⁸ highlights that it was expected that Neurology and Other revenue and profit growth would be driven in part by “orphan products.”³¹⁹ Mr. Pearson personally expected this growth.³²⁰ These expectations led to price increases early in 2014 on both drugs. The updated 2014 business plan for the unit implementing this mandate—as presented to Mr.

³¹³ Letter from Robert K. Kelner, Esq. to Samuel E. Dewey, Esq., at 4–5 (Mar. 22, 2016).

³¹⁴ See Email from Steven Sembler to J. Michael Pearson et al., VRX_SCA_00038631 (Sept. 11, 2013), and accompanying attachment, Neurology/Other Forecast 8 Update, VRX_SCA_00038645, at VRX_SCA_00038647 (Sept. 12, 2013); Pearson Deposition, at 181:17–22.

³¹⁵ See, *supra*, notes 291–92 and accompanying text (discussing Valeant’s robust bonus scheme).

³¹⁶ See, e.g., Neurology/Other Forecast 8 Update, VRX_SCA_00038645, at VRX_SCA_00038648 (Sept. 12, 2013); Pearson Deposition, at 182:22–183:9; First Set of Interrogatories to Robert Rosiello, at ¶ 8 and attached charts (Mar. 27, 2016, amended Aug. 12, 2016) (“Rosiello Interrogatories”).

³¹⁷ See Neurology/Other Forecast 8 Update, VRX_SCA_00038645, at VRX_SCA_00038648 (Sept. 12, 2013).

³¹⁸ See Email from Howard Schiller to Laura Gaibor and J. Michael Pearson, VRX_SCA_00459645 (Dec. 12, 2013), and accompanying attachment, 2014 Budget: Board of Directors Discussion, VRX_SCA_00459646 (Dec. 12, 2013).

³¹⁹ *Id.* at VRX_SCA_00459655.

³²⁰ See Pearson Deposition at 203:15–204:2.

Pearson, Mr. Schiller, and others—showed the extraordinary profitability generated by these price hikes.³²¹ In particular, the “Executive Summary” highlighted “Changes from 1st Submission in October,” including, “[m]ore aggressive pricing actions on Orphan products (Syprine and Cuprimine),” and a *reduction* in Valeant donations to the foundation providing patient assistance for these drugs.³²²

Turning to the latter part of 2014, the strategy of driving impressive performance relative to the budget (and reaping the resultant bonuses) continued.³²³ Prices also continued to increase.³²⁴

Into 2015, Neurology and Other’s November tracking plan indicated that the unit would reach a billion dollars in revenue in December 2014—three years ahead of schedule.³²⁵ This same plan called for massive growth in revenue, again fueled by substantial price increases.³²⁶ The update from the unit for the 2015 Board Budget review boasted:

- “Neuro Business Unit Striving to be 1st Valeant unit to achieve \$1B in annual revenue and will achieve double digit growth for revenue and EBITA for 2014.”³²⁷
- “Neuro Business Unit will continue to produce double digit growth for revenue and EBITA in 2015.”³²⁸

Valeant again took substantial price increases on Syprine and Cuprimine in 2015, presumably to meet these targets.³²⁹

³²¹ See Email from Steven Sembler to J. Michael Pearson, et al., VRX_SCA_00458596 (Nov. 11, 2013), and accompanying attachment, Neurology/Other 2014 Plan, VRX_SCA_00458610, at VRX_SCA_00458611 and VRX_SCA_00458614 (Nov. 12, 2013).

³²² *Id.* at VRX_SCA_00458611 (Nov. 12, 2013).

³²³ See Email from Steven Sembler to J. Michael Pearson et al., VRX_SCA_00336101 (July 2, 2014), and accompanying attachment, Neuro Q3 Projections & Outlook, VRX_SCA_00336102, at VRX_SCA_00336113–18 (July 7, 2014); Email from Steven Sembler to J. Michael Pearson et al., VRX_SCA_00151663 (Apr. 7, 2014), and accompanying attachment, Neuro Update 2014 Q1 FY14 and Full Year, VRX_SCA_00151664, at VRX_SCA_00151664–70 (Apr. 8, 2014).

³²⁴ See, *supra*, at 54.

³²⁵ See Email from Laura Gaibor to J. Michael Pearson, VRX_SCA_00056530 (Oct. 24, 2014), and accompanying attachment, Neuro Q 2014 Outlook & 2015 Plan, VRX_SCA_00056531, at VRX_SCA_00056533 (Nov. 10, 2014); see also, *supra*, at 51 (discussing the Unit’s 5 year business plan).

³²⁶ See Email from Laura Gaibor to J. Michael Pearson, VRX_SCA_00056530 (Oct. 24, 2014), and accompanying attachment, Neuro Q 2014 Outlook & 2015 Plan, VRX_SCA_00056531, at VRX_SCA_00056533, VRX_SCA_00056543 (Nov. 10, 2014).

³²⁷ Email from Laura Gaibor to J. Michael Pearson, VRX_SCA_00499052 (Nov. 12, 2014), and accompanying attachment, Neurology/Other Business 6Update, VRX_SCA_00499518, at VRX_SCA_00499536 (Nov. 20, 2014).

³²⁸ *Id.*

³²⁹ See, *supra*, at 54.

5. Aggressive Price Increases

Valeant knew that Wilson disease is a deadly disease. This stark reality, coupled with its control over the drugs that were essential for the well-being of most victims of the disease, gave Valeant monopoly “pricing power” over Cuprimine and Syprine. Valeant exploited this, as Mr. Pearson admitted in response to questioning at the April Committee hearing:

Senator Kaine: In your thinking about this free market system you are describing, is it a factor . . . [that] . . . the absence of Syprine could lead to liver failure or a liver transplant or even death? Is that a factor?

Mr. Pearson: It is³³⁰

a. Gold Standard

Health care professionals consider Syprine (and in the eye of a minority of providers, Cuprimine) to be the gold standard for treating Wilson disease.³³¹ Valeant knew that Syprine had largely displaced Cuprimine as the gold standard for Wilson disease treatment. As one executive put it in an email to Mr. Pearson “Cuprimine is considered an inferior product to Syprine so any loss is likely to Syprine which is ahead of QTD and YTD plan and is a higher priced product.”³³² As the Director of Business Development explained, when asked about a presentation Valeant prepared to share product opportunities with potential international partners, being the gold standard sells more drugs:

Q: “Strength: Market leader in the treatment of Wilson’s disease, chronic therapy with no generic forms on the market.” Why would you understand that to be a strength?

A: My understanding would be if you’re a market leader, that’s certainly good and usually means it’s preferred therapy that people want to use. Chronic therapy means that people need to continue to use it. And not having a generic would mean that there’s not competition.³³³

³³⁰ April 2016 Hearing, Trans. at. 97:17–23.

³³¹ See, *supra*, at 47–48.

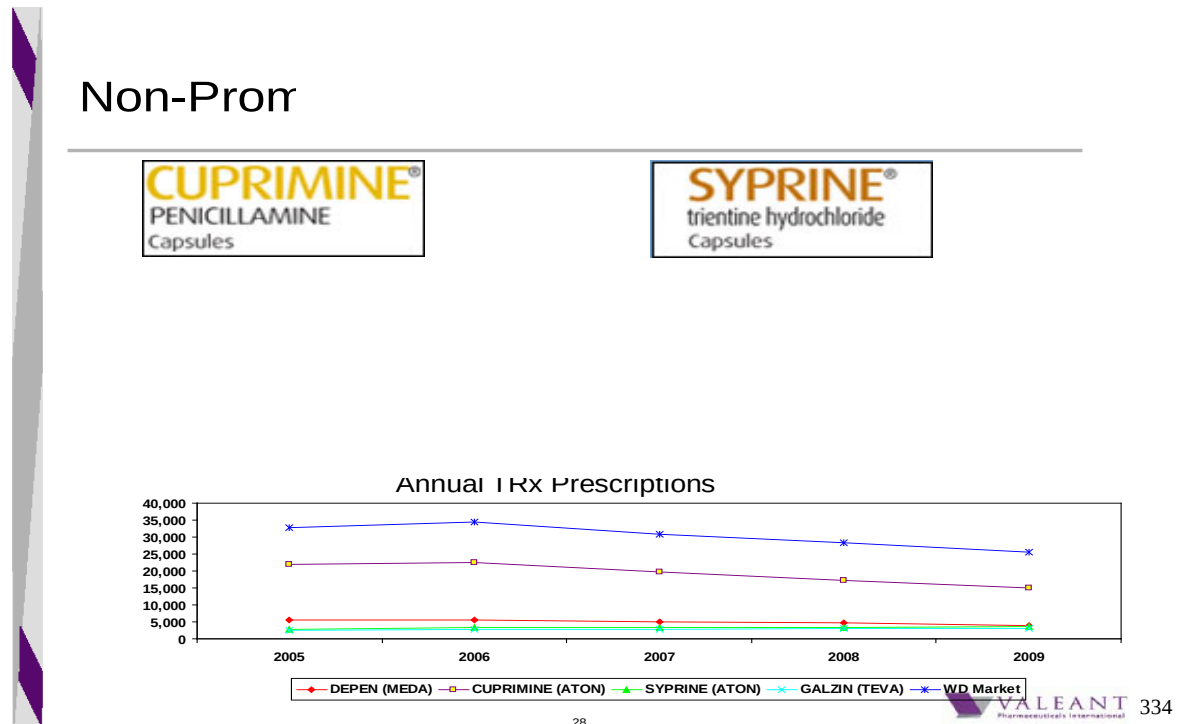
³³² Email from J. Michael Pearson to Rajiv DeSilva, VRX_SCA_00371892, at VRX_SCA_00371893 (June 13, 2011).

³³³ Davis Deposition, at 47:15–48:3 (quoting Email from Jeff Straus to Jonathan Ercole, VRX_SCA_00253149 (May 7, 2013), and accompanying attachment, BD Summit Orphan Products, VRX_SCA_00253150, at VRX_SCA_00253152 (undated)); see also, Davis Deposition, at 46:15–47:10 (discussing provenance of the BD Summit Orphan Products document).

b. Limited Substitutability

Even prior to acquiring Syprine and Cuprimine, Valeant focused on the fact that the drugs had limited substitutes and were effectively sole-source, as shown in **Figure 3**, a slide prepared for the acquisition.

Figure 3. Valeant Cuprimine and Syprine Acquisition Slide



The theme of exclusivity continued in subsequent presentations shared by Valeant executives, which noted that “Syprine is the only trientine hydrochloride available for the treatment of Wilson’s disease,”³³⁵ and that the company’s Orphan Drug Strategy was successful in part because Cuprimine and Syprine were “[o]rphan niche assets with few alternative therapies.”³³⁶

³³⁴ Electronic Meeting Invitation from J. Michael Pearson to Valeant Board of Directors, VRX_SCA_00284882 (Apr. 15, 2010), and accompanying attachment, Project Atom, Presentation to Board of Directors, VRX_SCA_00284883, at VRX_SCA_00284887, VRX_SCA_00284910 (Apr. 15, 2010).

³³⁵ Orphan Disease Produce Launch Plan, VRX_SCA_00055326, at VRX_SCA_00055342 (undated).

³³⁶ See, e.g., Email Steven Sembler to J. Michael Pearson et al., VRX_SCA_00038631 (Sept. 11, 2013), and accompanying attachment, Neurology/Other Forecast 8 Update, VRX_SCA_00038645, at VRX_SCA_00038654 (Sept. 12, 2013); Email from Steven Sembler to Scott Barry, VRX_SCA_00059485 (Oct. 7, 2013), and accompanying attachment, Neurology/Other 2014 Plan, VRX_SCA_00059486, at VRX_SCA_00059509 (Oct. 10, 2013).

c. Small Market

From the earliest exploration of the inherent “value” of Syprine and Cuprimine to Valeant, senior Valeant executives recognized that these small market drugs, serving a limited patient population, would provide Valeant with more pricing power.³³⁷ Mr. Pearson himself acknowledged this point at the Committee’s April hearing:

Senator Kaine. Do you understand—you know, we are using [the] phrase “orphan drugs.” Do you understand when we talk about this as basically a “patient as hostage” model, do you understand why we have come to look at it that way?

Mr. Pearson. I certainly have learned more today, and I do understand the description that you are giving now.³³⁸

Valeant also noted that price increases on orphan drugs had a “relatively small” overall impact on health budgets and was “largely tolerated by the medical community” for that reason.³³⁹

6. Patient Assistance Program Furthered Monopoly Profits

Valeant repeatedly touted its PAP, the VCPP, in response to criticisms of its Cuprimine and Syprine price increases. But based on internal Valeant documents, the Committee believes that these programs were driven not by altruism, but by Valeant’s desire to extract monopoly profits and then conceal that fact from the public.³⁴⁰

a. The Valeant Coverage Plus Program Was Designed to Increase Revenue

On a basic level, revenue in pharmaceuticals is determined by the variables of price (how much is charged for a drug), and volume (how many pills are sold). Co-pay assistance is often one way to increase revenue. For example, take a drug that costs \$100,000 for a pill. Suppose its net costs to the company that makes it (including SG&A and other allocated corporate overhead) is \$10,000.³⁴¹ Now suppose that insurance covers \$80,000 of the cost, but leaves a patient with a \$20,000 co-pay. In theory that copay would cause the patient to be sensitive to the cost of the drug and push back on price changes and high drug costs by changing their level of consumption, if possible. This pushback could lead to reduced volume, negating an increase in company profits from price increases. But when companies offer co-pay assistance so that the

³³⁷ See, e.g., Electronic Meeting Invitation from J. Michael Pearson to Valeant Board of Directors, VRX_SCA_00284882 (Apr. 15, 2010), and accompanying attachment, Project Atom, Presentation to Board of Directors, VRX_SCA_00284883, at VRX_SCA_00284884 (Apr. 15, 2010); see also Pearson Deposition, at 32:10–19 (noting that although Pearson was attracted to the transaction because of Aton’s ophthalmology assets, “[a] significance piece of Aton, in terms of revenue, was orphan”).

³³⁸ Senate Aging Committee Hearing, *Valeant Pharmaceutical’s Business Model: The Repercussions for Patients and the Health Care System*, Trans. at. 98:6–11 (Apr. 27, 2016).

³³⁹ Orphan Disease Product Launch Plan, VRX_SCA_00055326, at VRX_SCA_00055360 (undated).

³⁴⁰ To be sure, when pressed on these points, Mr. Pearson testified at the Committee’s hearing that “for patient assistance programs, we do not look at it as an investment with a return.” April 2016 Hearing, Trans. at. 116:15–17.

³⁴¹ See Appendix for an explanation of the terms of art used in drug pricing.

patient pays almost nothing, companies eliminate that vector for downward pressure on pricing. And the drug company still profits from the sale of drugs. Even if the drug company “pays” the entire copay (which is just an internal credit), it will still net a \$70,000 profit (the \$80,000 payment by insurance minus the \$10,000 cost of production).

As Senator Warren explained at the Committee’s April 2016 hearing, these economics suggest that patient assistance programs may be more about profit than charity:

Well, so because what is interesting to me about this is it means, if I am following the math right on this, you double the price even if you manage to give a waiver to the customer, you are still making a lot more money on this. And part of the way I figured this out is there is a Bloomberg report out that says that the pharmaceutical industry spent about \$7 billion on copay assistance in 2015, and that was up from \$1 billion in 2010. That all sounds pretty good until you get to the rest of the math.

According to multiple analyses, these programs actually benefit drug companies when alternatives may be available and shifting the costs of expensive drugs to consumers and to the insurance companies, so we all pay higher premiums in order to cover if the insurance company is still paying for it, and the drug companies are still picking up the money and putting it in their pockets.³⁴²

From the outset, the Valeant Coverage Plus Program was a poster child for the economic point raised by Senator Warren. It was about delivering co-pay assistance to the large percentage of Wilson disease patients on commercial insurance in order to increase volume and resulting profits.³⁴³ The “Orphan Disease Product Launch Plan” made clear that the program offered an “[o]pportunity to expand patient access and utilization with maximizing value. . . .”³⁴⁴ That presentation went on to state as benefits to Valeant:

- “Maximizes patient acquisition and retention by removing financial barriers.”³⁴⁵
- “Enhances per patient value through compliance and persistency.”³⁴⁶
- Allows for direct control of cost management programs.”³⁴⁷

Indeed, in 2014, an internal Valeant presentation listed a “Copay Card” not as a patient assistance initiative, but in a chart entitled “Summary *Marketing* Grid Supporting 2014/2015

³⁴² April 2016 Hearing, Trans. at. 115:19–116:10.

³⁴³ See Email from Hemanth Varghese to J. Michael Pearson and Howard Schiller, VRX_SCA_00039561 (Apr. 20, 2013), and accompanying attachments, Neurology/Other Forecast Five, VRX_SCA_00039562 (Apr. 22, 2013) and Neurology/Other Forecast Five-Backup, VRX_SCA_00039587 at VRX_SCA_00039629, VRX_SCA_00039635 (Apr. 22, 2013) (Noting commercial Cuprimine sales of 64.7 percent and Syprine sales of 69.30 percent).

³⁴⁴ Orphan Disease Product Launch Plan, VRX_SCA_00055326, at VRX_SCA_00055328 (undated).

³⁴⁵ *Id.* at VRX_SCA_00055332.

³⁴⁶ *Id.*

³⁴⁷ *Id.*

Strategies.”³⁴⁸ That presentation went on to list under “key *Marketing* Initiatives—Summary,” “Orphan Strategy,” “[i]ncrease Valeant Coverage Plus Program visibility through Wilsons Disease Association and direct mail campaigns to HCPs.”³⁴⁹ And the Neurology and Other 2015 Plan list a priority for VCPP:

- “Increase awareness and improve patient access to VCPP.”³⁵⁰
- “Maximize reimbursement while minimizing patient out of pocket expense on Orphan assets.”³⁵¹

Furthermore, an email from Mr. Kornwasser—one of the most senior of Valeant executives who was personally trusted by Mr. Pearson³⁵²—clearly demonstrates Valeant’s motive with the VCPP:

[May 19, 2014, 10:22 a.m. Clarissa Alvarado, Valeant Customer Services Lead (“Alvarado”) to Kornwasser]: Good morning Laizer: Cheryl asked me to send you the information we provide to callers who complain about the price of Syprine, Cuprimine or Targretin. We have a program for Syprine and Cuprimine called Valeant Coverage Plus. Targretin is part of the Valeant Patient Assistance Program, which is managed by Inventiv Health.

[10:41 a.m., Kornwasser to Alvarado]: Do we have the names of people that have called in regarding Cuprimine/Syprine/Targretin over the last 2 months?

[10:43 a.m., Alvarado to Kornwasser]: Unfortunately, we don’t have a way to store or track that information.

[10:45 a.m., Kornwasser to Alvarado]: For these 3 drugs we need to find a way asap.

[10:54 a.m., Alvarado to Kornwasser]: I believe that B&L Consumer Affairs uses Salesforce.com to track these types of complaints, but our team does not have access or training on this system.

[11:04 a.m., Kornwasser to Alvarado]: What do we need to do to be able to do this?

[11:13 a.m. Alvarado to Kornwasser]: Janice Glerum manages the group that has access. I will reach out to her to see if she can offer guidance on how our team can get access.

[11:15 a.m. Kornwasser to Alvarado]: *Thx. These patients are too valuable to lose.*³⁵³

³⁴⁸ Email from Laura Gaibor to J. Michael Pearson, VRX_SCA_00499052 (Nov. 12, 2014), *accompanying attachment*, Neurology/Other Business Update, VRX_SCA_00499518, at VRX_SCA_00499526 (Nov. 20, 2014) (emphasis added).

³⁴⁹ *Id.* at VRX_SCA_00499527.

³⁵⁰ Email from Sandeep Lalit to Steven Sembler, et al., VRX_SCA_00483659 (Sept. 4, 2014), *and accompanying attachment*, Neurology/Other 2015 Plan, VRX_SCA_00483660, at VRX_SCA_00483669 (Sept. 4, 2014).

³⁵¹ *Id.*

³⁵² Pearson Deposition, at Written Response 1.

³⁵³ Email from to Laizer Kornwasser to Clarissa Alvarado, VRX_SCA_00061484, at VRX_SCA_00061484-5 (May 20, 2014) (emphasis added).

b. Valeant’s Coverage Plus Program Was Designed to Mute Criticism from Patients and the Press

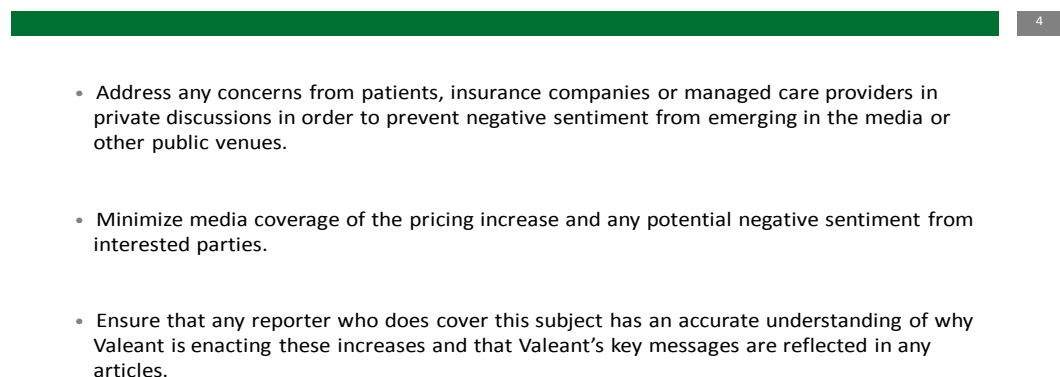
Valeant was acutely aware from the beginning that “[s]ubstantial price actions could attract undue negative publicity from patients, HCP’s, payers, and/or government agencies.”³⁵⁴ Accordingly, the VCPP was designed with the following objectives:

- “Privately address concerns from patients, insurance companies or managed care providers to prevent public displays of negative sentiment.”³⁵⁵
- “Minimize media coverage of the pricing increase.”³⁵⁶
- “Ensure understanding of why Valeant is enacting these increases and that Valeant’s key messages are reflected in any publications.”³⁵⁷

Another portion of the presentation, shown in **Figures 4–5**, echoed these points.

Figure 4. Valeant’s Coverage Plus Program Objectives

Strategic Objectives



³⁵⁴ Orphan Disease Product Launch Plan, VRX_SCA_00055326, at VRX_SCA_00055344 (undated).

³⁵⁵ *Id.* at VRX_SCA_00055346.

³⁵⁶ *Id.*

³⁵⁷ *Id.*

Figure 5. Valeant's Recommended Approach to Pricing Concerns

Recommended Approach

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- We recommend a reactive communications strategy designed to prevent Valeant from doing or saying anything that draws unwanted attention to the Wilson's disease drug rate increases.
- Valeant should use the following communications materials as a framework to respond reactively to concerns from patients and insurance companies/managed care providers, or inquiries from the media.
- Before the rate increases are enacted Valeant should develop a way to internally monitor, flag and escalate any patient, insurer, or managed care provider concerns that arise.
- Jeff Strauss or a member of his team should notify Laurie Little and the Sard Verbinen team of any media inquiries that are received on this subject.
- If concerns gain traction in the medical community or media, we would recommend hosting a working group conference call to discuss if the communications approach needs to be altered in any way to address specific issues.



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This bent—use free drugs and generous patient assistance to minimize criticism—continued in internal documents circulated among senior Valeant executives. Shortly after release, Valeant's Coverage Plus Program was touted to senior executives for its role in ensuring that "[p]ositive patient experience increases persistency & minimizes pricing concerns."³⁵⁹ In addition, part of the Neurology and Others Unit's 2015 budget planning was to "[c]ontinue to monitor social media outlets for patient feedback (blogs) related to product cost and access in order to mitigate potential for negative PR."³⁶⁰

c. Price Gouging

Profit motive drove the pricing strategy for Cuprimine and Syprine, and Valeant made extraordinary profits. Sworn interrogatory responses from Valeant's then-CFO Mr. Rosiello indicate that since the implementation of the price increases, Valeant made massive profits, these profits drove corporate profitability, Valeant paid almost nothing in cost of goods or otherwise to make the drugs, and Valeant did not invest a cent in research and development on those drugs.³⁶¹

³⁵⁸ *Id.* at VRX_SCA_00055361–62.

³⁵⁹ Email from Steven Sembler to J. Michael Pearson, et al., VRX_SCA_00038631 (Sept. 11, 2013), and accompanying attachment, Neurology/Other Forecast 8 Update, VRX_SCA_00038645, at VRX_SCA_00038655 (Sept. 12, 2013).

³⁶⁰ Email from Laura Gaibor to J. Michael Pearson, VRX_SCA_00056530 (Oct. 23, 2014) Neuro Q 2014 Outlook & 2015 Plan, VRX_SCA_00056531, at VRX_SCA_00056560 (Nov. 10, 2014).

³⁶¹ See Rosiello Interrogatories, at ¶ 8 and attached charts, ¶ 5 (noting that Valeant did not record any Research and Development expenses for Syprine or Cuprimine), and ¶¶ 6 and 7 (detailing the methodology by which certain numbers were calculated)

	2013				2014				2015				2016	
	Q1	Q2	Q3	Q4	Q1	Q2	Q3	Q4	Q1	Q2	Q3	Q4	Q1	Q2
I. Cuprimine														
Gross Product Sales, in U.S. Dollars*	\$2.33	\$3.18	\$6.03	\$7.69	\$9.72	\$10.11	\$16.30	\$20.05	\$14.58	\$9.86	\$50.79	\$52.14	\$55.68	\$50.62
Net Product Sales, in U.S. Dollars*	\$1.92	\$2.41	\$6.00	\$5.87	\$7.69	\$7.27	\$10.98	\$10.85	\$8.34	\$8.00	\$27.06	\$26.71	\$27.20	\$25.43
Cost of Goods Sold, in U.S. Dollars*	\$0.14	-\$0.03	\$0.04	-\$0.15	\$0.07	\$0.24	\$0.24	\$0.21	\$0.14	\$0.10	\$0.28	\$0.18	\$0.08	\$0.21
Net Income Attributable to Cuprimine in U.S. Dollars*	\$1.41	\$2.07	\$5.59	\$5.65	\$6.92	\$6.33	\$10.03	\$9.93	\$6.74	\$6.43	\$25.31	\$25.06	\$24.90	\$22.99
The Ratio of Cuprimine Net Sales to Net Product Sales of Valeant*	0.18%	0.22%	0.39%	0.28%	0.41%	0.36%	0.54%	0.49%	0.38%	0.29%	0.97%	0.97%	1.15%	1.05%
The Ratio of Cuprimine Net Income to the Net Income of Valeant*	0.33%	0.47%	32.71%	1.04%	1.15%	0.87%	0.99%	0.81%	0.80%	0.69%	2.32%	2.63%	3.30%	2.33%
2. Syprine														
Gross Product Sales, in U.S. Dollars*	\$3.32	\$3.94	\$7.67	\$28.68	\$24.81	\$25.55	\$29.36	\$48.61	\$29.83	\$35.01	\$32.35	\$40.28	\$44.45	\$36.30
Net Product Sales, in U.S. Dollars*	\$2.66	\$3.02	\$10.59	\$20.65	\$18.52	\$18.33	\$20.32	\$30.65	\$18.31	\$28.35	\$19.00	\$23.15	\$23.05	\$19.51
Cost of Goods Sold, in U.S. Dollars*	\$0.45	\$0.26	\$0.59	\$0.42	\$0.38	\$0.41	\$0.10	\$0.38	\$0.03	\$0.22	\$0.34	\$0.30	\$0.13	\$0.79
Net Income Attributable to Cuprimine in U.S. Dollars*	\$1.37	\$1.91	\$9.16	\$19.39	\$16.46	\$16.24	\$18.54	\$28.59	\$16.42	\$26.27	\$16.81	\$20.99	\$21.13	\$16.92
The Ratio of Cuprimine Net Sales to Net Product Sales of Valeant*	0.25%	0.28%	0.69%	1.00%	0.98%	0.90%	0.99%	1.37%	0.84%	1.04%	0.68%	0.84%	0.97%	0.81%
The Ratio of Cuprimine Net Income to the Net Income of Valeant*	0.32%	0.44%	53.60%	3.55%	2.72%	2.22%	1.83%	2.33%	1.94%	2.82%	1.54%	2.20%	2.80%	1.72%

*Dollars in Millions

Table 3. Valeant's Cuprimine and Syprine Profits

C. Isuprel and Nitropress

1. Background

Isuprel and Nitropress are both drugs that are designed to serve life-critical cardiac functions in hospital settings and have been on the market for decades:

- **Isuprel (isoproterenol)** (patented in 1956) is an injectable drug that relaxes blood vessels and helps the heart pump blood more efficiently. It is used in cases of cardiac arrest and can be life-saving during emergencies for patients with very slow heart rates. Isuprel is the standard of care in certain cardiac situations.³⁶²
- **Nitropress (nitroprusside)** (the active ingredient in this drug was first isolated in the 19th Century)³⁶³ is an injectable drug that reduces blood pressure in cases of cardiac emergencies.³⁶⁴ Nitropress is also a life-saving standard of care, although hospitals are now developing alternatives that may work in some cases.³⁶⁵

2. Acquisition

Valeant acquired Isuprel and Nitropress, and other drugs, from Marathon Pharmaceuticals (“Marathon”) in February 2015, for \$350 million, plus an estimated \$3.3 million in acquisition expenses and related costs.³⁶⁶ The same day it acquired these drugs, Valeant raised the price of Isuprel by 500 percent, from \$2,183.00 to \$13,097.10 for ten 5 milliliter vials and the price of Nitropress by 200 percent, from \$2,148.30 to \$6,444.90 for ten 2 milliliter vials.³⁶⁷

Valeant has since raised the price of Isuprel to \$17,901.12 for ten 5 milliliter vials, representing a 720 percent cumulative increase. It also raised the price of Nitropress to \$8,808.80 for ten 2 milliliter vials, representing a 310 percent cumulative increase.³⁶⁸

³⁶² See April 2016 Hearing, at 3–4 (written testimony of Richard Fogel, M.D.).

³⁶³ See *Developments in the Prescription Drug Market: Oversight: Hearing Before H. Comm. on Oversight and Gov’t Reform*, at 4 (Feb. 4, 2016) (written testimony of Howard B. Schiller).

³⁶⁴ See April 2016 Hearing, at 3 (written testimony of Richard Fogel, M.D.).

³⁶⁵ See April 2016 Hearing, at 3–4 (written testimony of Richard Fogel, M.D.). The FDA approved a generic version of Nitropress on December 9, 2016. FDA, *Drugs@FDA: FDA Approved Products*, found at, <http://www.accessdata.fda.gov/scripts/cder/daf/index.cfm?event=overview.process&ApplNo=207426> (last visited Dec. 12, 2016).

³⁶⁶ See Letter from Robert K. Kelner, Esq. to Samuel Everett Dewey, Esq., at 4 (Dec. 2, 2015).

³⁶⁷ See Letter from Robert K. Kelner, Esq. to Samuel Everett Dewey, Esq., at 4–5 (Mar. 22, 2016); Brady Dennis, *Rattled by Drug Price Increases, Hospitals Seek Ways to Stay on Guard*, The Washington Post (Mar. 13, 2016) found at, https://www.washingtonpost.com/national/health-science/rattled-by-drug-price-increases-hospitals-seek-ways-to-stay-on-guard/2016/03/13/1c593dea-c8f3-11e5-88ff-e2d1b4289c2f_story.html?utm_term=.436a50994307 (last visited Dec. 15, 2016). Isuprel is sold in packages of ten and Nitropress is sold in packages of one.

³⁶⁸ *Id.*

3. Monopoly Profits

The entire business case for the acquisition of Isuprel and Nitropress rested on acquiring a “mispriced” drug to raise its price dramatically.

Valeant began to explore acquiring a suite of drugs from Marathon late in 2014 after Marathon reached out to Mr. Schiller.³⁶⁹ The driving factor behind the transaction was the ability to take price increases on Isuprel and Nitropress, which represented the bulk of the transaction. A senior Valeant executive stated at the outset of evaluating the transaction, “this would also have to be a *price play* (if we determine there is upside to take price) as we don’t have a sales team calling on hospitals (ie no direct promotion).”³⁷⁰ Internal Valeant documents reflect a focus on price increases. As the Company’s consultant McKinsey & Co. (“McKinsey”) put it, “[a]ttached please find the findings on hospital pricing. In a nutshell, most of the products reviewed (Marathon, [REDACTED], and VRX) . . . have *material pricing potential*.”³⁷¹

Valeant made an initial offer to Marathon on December 19, 2014,³⁷² which was countered by Marathon at a higher purchase price.³⁷³ Marathon justified the higher purchase price by indicating that there was room for further price increases (Marathon had already taken a substantial price increase earlier in the year). Mr. Pearson testified that, “as part of Marathon’s pitch to us, they said that they still felt that there was a lot of—the products were still mispriced.”³⁷⁴ Mr. Davis further explained to Mr. Pearson, Mr. Schiller, and others, that Marathon provided Valeant with a pricing analysis commissioned by Marathon to demonstrate that Valeant had room to take more substantial price increases.³⁷⁵

After the transaction closed, Mr. Davis worked with Valeant’s auditors at Deloitte to put together an accounting for the deal, and answered questions posed by the auditors.³⁷⁶ Deloitte asked “[w]hat was the motivation for the transaction (ability to take price, low promotion

³⁶⁹ See Schiller Deposition, at 194:10–195:6.

³⁷⁰ Email from Andrew Davis to Steven Sembler et al., VRX_SCA_00001104, at VRX_SCA_00001104 (Dec. 3, 2014) (emphasis added).

³⁷¹ Email from Andrew Davis to Steven Sembler et al., VRX_SCA_00001012 (Dec. 29, 2014) (emphasis added).

³⁷² See Non-Binding Letter of Intent Between Valeant Pharmaceuticals Int. Inc. and Marathon Pharmaceuticals, LLC, VRX_SCA_000786 (Dec. 19, 2014); Email from Andrew Davis to Barbar Ghias (Marathon), VRX_SCA_00016485, at VRX_SCA_00016485 (Dec. 12, 2014).

³⁷³ See Email from Babar Ghias to Andrew Davis and Alex Matheson, VRX_SCA_00014125, at VRX_SCA_00014125 (Dec. 31, 2014).

³⁷⁴ Pearson Deposition, at 244:13–16.

³⁷⁵ See Email from Andrew Davis, to J. Michael Pearson, et al., VRX_SCA_00593925, at VRX_SCA_00593925 (Dec. 30, 2014) (“Fyi, attached is the pricing report Marathon had done ahead of the acquisition. They have also said that the same folks did an informal review this year . . . that said there was a further pricing upside.”). See also, Email from Andrew Davis to Laurie Little and Tanya Carro, VRX_SCA_00230933, at VRX_SCA_00230933 (Mar. 23, 2015) (“the seller hired a pricing consultant ahead of their sale to be able to provide some of the detail on the product price potential”). The mere fact that Marathon forwarded the report to Valeant is significant. Marathon refused to produce the unredacted report to the Committee on the grounds that it was business sensitive and refused to consent to Pennside discussing the report with the Committee.

³⁷⁶ See Davis Deposition, at 122:16–123:3.

cost)?”³⁷⁷ Mr. Davis answered, “[t]ail end products where there was opportunity to promote and price efficiently” (with “price efficiently” meaning take price increases).³⁷⁸ Valeant’s July 2015 price increases on Isuprel appear to have been motivated by a desire meet Pearson’s apparent desire to improve projected numbers.³⁷⁹

Confronted with evidence during the Committee’s hearing, Mr. Pearson backed down from Valeant’s prior external statements³⁸⁰ and confessed: “In hindsight, I regret pursuing transactions where *the central premise was based on an increase in price*, for example, our acquisition of Nitropress and Isuprel from Marathon.”³⁸¹

a. Sole-Source & Gold Standard

From the beginning, Valeant planned to take aggressive price increases on Isuprel and Nitropress because the products were sole-source, the gold standard, and had limited substitutability. In evaluating the transaction, Valeant commissioned a study from McKinsey on Isuprel and Nitropress,³⁸² which, among other things, reported on the “pricing potential” of these drugs.³⁸³ McKinsey determined Isuprel had a high level of “pricing potential” because it was “[c]onsidered the standard of care” and “must be available in limited situations where needed.”³⁸⁴ McKinsey elaborated that there was “[n]o therapeutically equivalent generic version of Isuprel available in the United States.”³⁸⁵ McKinsey assigned even higher pricing potential to Nitropress, as it was “[c]onsidered the clear differentiation from alternatives (e.g., fastest acting half-life),” and the “product must be available in limited situations where needed.”³⁸⁶ In supporting materials, McKinsey noted that “Both [drugs] are standard of care drugs which must be available for a small proportion of cases when required by physician.”³⁸⁷

³⁷⁷ Email from Andrew Davis to George Gadkowski, VRX_SCA_00002081 (Mar. 19, 2015), and accompanying attachment, Word Document, VRX_SCA_00002082 (Mar. 19, 2015).

³⁷⁸ See Davis Deposition, at 123:14–124:2.

³⁷⁹ Email from Brian Stolz to J. Michael Pearson and Tanya Carro, VRX_SCA_00101078 (July 21, 2015); see also, Email from Crag K. Olson, to J. Michael Pearson et al., VRX_SCA_00425447, and accompanying attachment, Neuro Q3 Discussion, VRX_SCA_00425448 (July 29, 2015).

³⁸⁰ See, *infra*, at 73–74.

³⁸¹ April 2016 Hearing, Trans. at 49:3–6 (emphasis added).

³⁸² Email from Andrew Davis to Steven Sembler, et al., VRX_SCA_00001012 (Dec. 29, 2014), and accompanying attachment, Hospital Product Pricing Insights, VRX_SCA_00001013 (Dec. 29, 2014).

³⁸³ Email from Andrew Davis to Steven Sembler, et al., VRX_SCA_00001012 (Dec. 29, 2014), and accompanying attachment, Hospital Product Pricing Insights, VRX_SCA_00001013, at VRX_SCA_00001017 (Dec. 29, 2014). McKinsey told the Committee that the presentation was more in the nature of market research versus a complete pricing analysis. Committee Staff Interview of Aamir Malik (McKinsey & Co.) (Apr. 19, 2016). This is a distinction without a difference. While the Committee does not doubt McKinsey could have conducted a more complete pricing analysis, the analysis they did conduct relates to, and was used by, Valeant for pricing.

³⁸⁴ Email from Andrew Davis to Steven Sembler, et al., VRX_SCA_00001012 (Dec. 29, 2014), and accompanying attachment, Hospital Product Pricing Insights, VRX_SCA_00001013, at VRX_SCA_00001017 (Dec. 29, 2014).

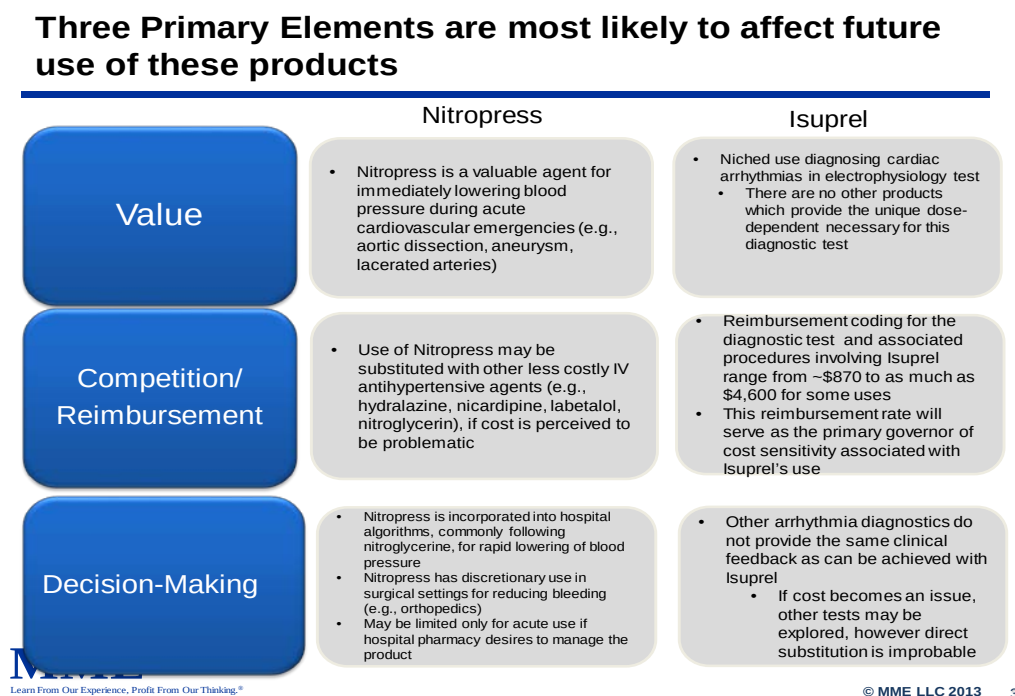
³⁸⁵ *Id.* at VRX_SCA_00001018.

³⁸⁶ *Id.* at VRX_SCA_00001017; see also *id.* at VRX_SCA_00001018.

³⁸⁷ *Id.* at VRX_SCA_00001019. McKinsey noted that in some circumstances, hydralazine was preferred over Isuprel, but McKinsey did not consider this significant because hydralazine was in shortage. *Id.* at VRX_SCA_00001037.

Valeant also relied upon a pricing analysis commissioned from MME (the company that had conducted the previous Marathon pricing report).³⁸⁸ This report echoed the conclusions of the McKinsey study, as shown in **Figure 6**.

Figure 6. MME Pricing Analysis of Nitropress and Isuprel³⁸⁹



Valeant admitted that the nature of Isuprel and Nitropress as sole-source, gold standard drugs drove the transactions. When Deloitte asked Mr. Davis “[w]hat are the competitive advantages for Nitropress and Isuprel,” he replied “[s]ole source products that have specific use in treatment paradigm.”³⁹⁰ Mr. Davis testified:

“By ‘sole source,’ I meant that there are no generics, they’re the only options available. And by ‘that have specific use in the treatment paradigm,’ from the research we had done or had done for us, we saw that they had—that they were specifically used in certain treatment—you know, in certain parts of the treatment paradigm where they had a specific role to play.”³⁹¹

³⁸⁸ The report’s “Objective[s]” were to “[r]eview the potential upward pricing flexibility for Nitropress . . . and Isuprel . . .” as well as to “[a]dvise on available pricing flexibility for both products.” Email from Steven Sembler to Laizer Kornwasser, VRX_SCA_0012784 (Jan. 20, 2015), and accompanying attachment, *Nitropress and Isuprel Pricing Flexibility Review Presented to Valeant*, VRX_SCA_00012785, at VRX_SCA_00012787 (Dec. 24, 2015).

³⁸⁹ *Id.* at VRX_SCA_00012788.

³⁹⁰ Email from Andrew Davis to George Gadkowski, VRX_SCA_00002081 (Mar. 19, 2015), and accompanying attachment, Word Document, VRX_SCA_00002082 (undated).

³⁹¹ Davis Deposition, at 124:9–17.

b. Small Market

Pre-transaction diligence makes clear that Valeant saw pricing potential in part due to the fact that Isuprel and Nitropress have small markets. Both are used in hospitals in emergency settings, limiting the extent to which price increases would attract attention and pushback from patients and insurance companies. McKinsey analyzed the extent to which a price increase would draw the attention of the hospital committees that control hospital drug purchases (often called a “P&T committee”). McKinsey concluded that hospitals would pay little attention to price increases on Isuprel or Nitropress, noting that “P&T committees have not focused on the product[s] . . . therapeutics class,”³⁹² that “[t]ypically, P&T committees focus on high cost/new therapies which could be misused,”³⁹³ “[p]roducts have been in the system for so long that reviews are practically rubber stamped,”³⁹⁴ “[r]eview processes for these products is to conduct a class review; answer is always to keep on formulary unless new clinical data,” “[r]ecent price increases have been relatively unnoticed, although few responded mentioned usage change,”³⁹⁵ and “11/12 respondents did not discuss recent price increases despite being provided context.”³⁹⁶

c. Slow Generic Approval

The Committee’s investigation also revealed that Valeant’s price-gouging strategy was directly tied to how difficult it would be for a generic competitor to enter the market. Isuprel and Nitropress were unique among the drugs that the Committee studied in that from the very beginning, Valeant knew ANDAs had been filed for both drugs and expected that at some point those ANDAs would be approved.³⁹⁷ This expectation did not deter Valeant because the Company calculated that FDA processing delays would still yield several years of de facto monopoly—more than enough time to generate massive profits.

Prior to the transaction, Valeant commissioned Pennside Partners, Ltd. (“Pennside”) to conduct a study of when generic entry could be expected.³⁹⁸ Pennside reported that ANDAs had been filed, and predicted delayed entry due to FDA backlogs:

³⁹² Email from Andrew Davis to Steven Sembler, et al., VRX_SCA_00001012 (Dec. 29, 2014), *and accompanying attachment*, Hospital Product Pricing Insights, VRX_SCA_00001013, at VRX_SCA_00001017 (Dec. 29, 2014).

³⁹³ *Id.* at VRX_SCA_00001019.

³⁹⁴ *Id.*

³⁹⁵ *Id.*

³⁹⁶ *Id.*

³⁹⁷ See Email from Andrew Davis to Steve Sembler et al., VRX_SCA_00001104, at VRX_SCA_00001104 (Dec. 4, 2014). Cf. Email from Andrew Davis to Babar Ghias, VRX_SCA_00016485, at VRX_SCA_00016485 (Dec. 12, 2014).

³⁹⁸ See Davis Deposition, at 67:20–68:2.

Figure 7. Valeant's Consultant Presentation on Generic Entry Potential

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Executive Summary

- X-Gen Pharma has filed ANDAs for both ISUPREL ampoules and NITROPRESS vials in June of 2014 and is expecting approval for both in the 2015-2016 time period under GDUFA II regulations
 - Pennside believes approval in late 2016 or 1H 2017 is more realistic
- Par Pharma Sterile Products filed an ANDA for NITROPRESS vials in June of 2014 and expects and also approval in the 2015-2016 time period under GDUFA II regulations, and it filed ISUPREL ampoules in Q1 2015 and is expecting approval under GDUFA III regulation in 2016
 - Pennside believes approval in late 2016 or 1H 2017 is more realistic for NITROPRESS
 - If Par does complete its filing in for ISUPREL in Q1 2015, an approval in 1H 2016 is highly possible (see Regulatory discussion on Page 8)
- Sandoz and Sagent have both also filed ANDAs in June of 2014 for NITROPRESS vials and both expect approval in the 2015-2016 time period under GDUFA II rules
 - Again, Pennside believes approval in late 2016 or 1H 2017 is more realistic

OraPharma/Valeant should assume there will be two (2) generic sources for ISUPREL and four (4) for NITROPRESS by mid-2017, with more to follow

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Pennside reported further details of FDA approval times on ANDA submissions and noted that “FDA Average Review Time for ANDA’s is 36–48 Months.”⁴⁰⁰

Mr. Sembler forwarded this presentation to Mr. Davis with the following instructions: “From this research, it looks like there could be more than one generic entry in the 2016/2017 timeframe. I believe this event would occur sooner than business model assumptions. We should take this risk into consideration with our offer.”⁴⁰¹

Valeant subsequently adjusted its early internal modeling, which had been predicated on generic entry in 2018.⁴⁰² Valeant did not pull out of the transaction, but swiftly calculated whether the few years of monopoly power under Pennside’s predictions were sufficient to generate immense returns.⁴⁰³ These revised calculations showed that the size of a potential price increase would not affect the date of generic entry (generally a higher price point makes a market

³⁹⁹ Email from Steven Sembler to Andrew Davis, VRX_SCA_00012705, and accompanying attachment, Pennside Partners, Ltd., *Isuprel & Nitropress Generic Threats in the U.S.*, VRX_SCA_00012706, at VRX_SCA_00012708 (Dec. 26, 2014).

⁴⁰⁰ *Id.* at VRX_SCA_00012713.

⁴⁰¹ Email from Steven Sembler to Andrew Davis, VRX_SCA_00012705, and accompanying attachment, Pennside Partners, Ltd., *Isuprel & Nitropress Generic Threats in the U.S.*, VRX_SCA_00012706, at VRX_SCA_00012708 (Dec. 26, 2014).

⁴⁰² See Email from Andrew Davis to Steven Sember, et al., VRX_SCA_00396915 (Dec. 29, 2014).

⁴⁰³ See, e.g., Pearson Deposition, at 222:7–14 (“**Q:** Here it says, Either way, the outside firm is expecting approvals in late 2016 through 2017. If approvals had been expected months after the transaction, would you have gone ahead with the transaction? **A:** Again, it would all depend, at that point in time, on the economics. . .”).

more attractive for a generic manufacturer to seek to enter the market) because ANDAs had already been filed for both products and the delay was entirely “a function of the backlog at the FDA.”⁴⁰⁴

Valeant executives were presented with a modeling metric with two variables, the length of the FDA delay in approving generic entrants and the amount of Valeant’s initial price increase, showing that Valeant had multiple paths to a viable transaction, shown in **Figure 8**.⁴⁰⁵

Figure 8. Valeant’s Modeling Matrix

	IRR/Payback	Day 1 increase				
		20%	30%	40%	50%	60%
Gx entry Nitropress and Isuprel	2015	15% IRR 9.1 Years	16% IRR 8.6 Years	17% IRR 8.1 Years	18% IRR 7.7 Years	20% IRR 7.3 Years
	2016	16% IRR 8.1 Years	18% IRR 7.5 Years	19% IRR 6.9 Years	21% IRR 6.4 Years	23% IRR 5.9 Years
	2017	18% IRR 6.7 Years	20% IRR 5.9 Years	22% IRR 5.2 Years	24% IRR 4.5 Years	26% IRR 3.9 Years
	2018	20% IRR 4.7 Years	22% IRR 3.8 Years	24% IRR 3.3 Years	27% IRR 2.9 Years	29% IRR 2.8 Years
	2019	21% IRR 3.7 Years	24% IRR 3.4 Years	26% IRR 3.2 Years	29% IRR 2.9 Years	31% IRR 2.8 Years
	2020	23% IRR 3.7 Years	25% IRR 3.4 Years	28% IRR 3.2 Years	31% IRR 2.9 Years	33% IRR 2.8 Years

⁴⁰⁶

Davis and others performed analyses to determine how to profit from the FDA’s delays in processing generic applications, including discussing with Pennside “the realities of the agency speeding up approval for Generic drugs (which they have committed to do, but which hasn’t actually occurred yet).”⁴⁰⁷

1. Price Gouging

As with Syprine and Cuprimine, Valeant’s profits from raising prices for Isuprel and Nitropress were extraordinary. Post-transaction, Deloitte asked Mr. Davis if he was “ok” with certain financial assumptions, noting those assumptions “are leading to high gross margins (more than 99%).”⁴⁰⁸ Mr. Davis responded, “Standard cost looks right, and I’m not surprised they are

⁴⁰⁴ Schiller Deposition, at 209:14–22.

⁴⁰⁵ Valeant evaluated its transactions by two metrics, IRR and the time to payback. See Pearson Deposition, at 223:21–224:5. The first metric reflects the projected positive return on the investment adjusted for present day values. See Davis Deposition, at 64:6–14, 65:8–9. The second reflects when the cost of the purchase has been recouped assuming free cost of money. See Pearson Deposition, at 224:3–5. As a general proposition, Valeant would only engage in transactions with an IIR of at least 20 percent and a payback period of six years or less, with the criteria adjusted somewhat for risk. *Id.* at 224:7–15.

⁴⁰⁶ Email from Andrew Davis to J. Michael Pearson and Howard B. Schiller, VRX_SCA_00593866, at VRX-SCA_00593866 (Dec. 26, 2014).

⁴⁰⁷ *Id.*

⁴⁰⁸ Email from Andrew Davis to George Gadkowski, VRX_SCA_00016376 (Mar. 2015).

extremely profitable.”⁴⁰⁹ In interrogatory responses provided to the Committee, Valeant admitted that it spent nothing on Research and Development for Isuprel and Nitropress since acquisition. Valeant also admitted that its net income from the four drugs dwarfs the manufacturing cost. The margins were extraordinary, as exhibited in **Table 4**.⁴¹⁰

Table 4. Valeant’s Profit from Isuprel and Nitropress

	2015				2016	
	Q1	Q2	Q3	Q4	Q1	Q2
1. Isuprel						
Gross Product Sales, in U.S. Dollars*	\$107.44	\$46.50	\$63.86	\$89.52	\$117.98	\$82.12
Net Product Sales, in U.S. Dollars*	\$72.22	\$48.90	\$49.78	\$53.40	\$65.93	\$39.67
Cost of Goods Sold, in U.S. Dollars*	\$0.07	\$0.08	\$0.03	\$0.20	\$0.09	\$0.10
Net Income Attributable to Isuprel in U.S. Dollars*	\$69.22	\$43.54	\$44.47	\$47.92	\$61.38	\$35.10
The Ratio of Isuprel Net Sales to Net Product Sales of Valeant*	3.33%	1.79%	1.79%	1.94%	2.78%	1.64%
The Ratio of Isuprel Net Income to the Net Income of Valeant*	8.19%	4.67%	4.07%	5.03%	8.13%	3.56%
2. Nitropress						
Gross Product Sales, in U.S. Dollars*	\$76.18	\$56.51	\$39.34	\$68.11	\$71.18	\$52.01
Net Product Sales, in U.S. Dollars*	\$61.58	\$64.23	\$35.25	\$57.69	\$58.02	\$34.39
Cost of Goods Sold, in U.S. Dollars*	\$0.18	\$0.28	\$0.09	\$0.80	\$0.61	\$0.54
Net Income Attributable to Nitropress in U.S. Dollars*	\$58.54	\$58.81	\$30.01	\$51.74	\$53.50	\$29.94
The Ratio of Nitropress Net Sales to Net Product Sales of Valeant*	2.84%	2.35%	1.26%	2.09%	2.45%	1.42%
The Ratio of Nitropress Net Income to the Net Income of Valeant*	6.92%	6.31%	2.75%	5.43%	7.09%	3.04%

*Dollars in millions; Data prior to Q1 of 2015 is not applicable.

Even more revealing is that these massive price increases were not necessary to provide substantial profits. A far lower price increase would have provided a desirable return. Mr. Schiller confirmed this during the Committee’s April 2016 Hearing:

⁴⁰⁹ Email from Andrew Davis to George Gadkowski, VRX_SCA_00016376 (Mar. 2015).

Id. Mr. Davis explained that his use of “extremely” was based on the fact that the transaction was already profitable and the accounting treatment Deloitte accorded to the transaction made it “extremely profitable.” Davis Deposition, at 137:10–138:9.

⁴¹⁰ Rosiello Interrogatories and responses, at ¶ 8 and attached charts, and ¶ 5 (noting that Valeant did not record any Research and Development expenses for Isuprel and Nitropress), and ¶¶ 6 and 7 (detailing the methodology by which certain numbers were calculated).

Chairman Collins: I want to better understand why your company felt it was necessary to take such substantial price increases on Isuprel and Nitropress. Valeant built a model—and I have seen the model—to project whether the acquisition would meet certain metrics of profitability, and then that model is used as a major tool in determining whether or not to complete the transaction, in this case to buy the two drugs.

Mr. Schiller, it is my understanding that the model found that the transaction would be viable financially for Valeant at a 60-percent increase. That is what was reflected in the deal model. Is that correct?

Mr. Schiller: I do not recall the specifics in the matrix that I was shown in my deposition, but it was certainly lower than the ultimate price increase that was taken.

Chairman Collins: Well, that is according to the deposition from Mr. Andrew Davis, and would you have any reason to doubt his sworn testimony?

Mr. Schiller. I would not.

Chairman Collins: So Valeant could have been profitable with acquiring these two drugs and raising the price by 60 percent. That is still a substantial price increase, but it is far different from the price increase that ultimately was taken. Could you explain why the price was so much higher than the 60 percent that was recommended in the model? Yes, Mr. Schiller.

Mr. Schiller: There was a meeting that a number of us attended—Mr. Pearson, myself, Andrew Davis you mentioned in the business unit. They reviewed the findings of the consulting firm. They made a recommendation which was lower than that price, and Mr. Pearson made a decision to go with the higher price increase.⁴¹¹

D. Company-Wide Monopoly Strategy

Under oath, Mr. Pearson and other Valeant Executives repeatedly claimed the Neurology and Others Unit was the only unit employing an aggressive strategy of price increases and that Neurology and Others represented a small portion of Valeant's overall business. The Committee found otherwise on both claims.

As Senator McCaskill illustrated during the Committee's hearing, the strategy of price increases took place company-wide:

- **Sen. McCaskill:** According to your SEC filings, Mr. Pearson, beginning in the first quarter of 2013 through the third quarter of 2015, you state in your filings that your revenue—changes in revenue have been driven primarily by price, not by growth. In fact, in only one quarter between 2013 and 2015 did you report that growth was driven by volume. So price increases has, in fact, been the entree for your business, correct?

Mr. Pearson: Yes, pricing has driven more growth than volume, although that is changing over time.⁴¹²

⁴¹¹ April 2016 Hearing, Trans. at. 120:21–122:4.

⁴¹² *Id.* at. 66:24–67:8.

- **Senator McCaskill:** Can you find me one drug that Valeant did not raise the price on?
Mr. Ackman. I do not know offhand the price—I do not have the price list.
Senator McCaskill: Mr. Pearson, one drug that you did not raise the price on after you acquired it?
Mr. Pearson: Not in the United States.
Senator McCaskill: Mr. Schiller, are you aware of any drug that you bought or acquired that you did not raise the price on?
Mr. Schiller: My recollection is when we bought Salix, we did not raise the price on Xifaxan.⁴¹³

Additionally, Valeant’s repeated assertions that the Neurology and Other unit represented a small part of Valeant’s total business are also contradicted by the company’s financial data and internal documents. In an email from Mr. Schiller to Mr. Pearson, he notes that “[l]ast night, one of the investors asked about price vs. volume for Q1. Excluding marathon, price represents about 60% of our growth. If you include marathon, price represents about 80.”⁴¹⁴ Additionally, Valeant’s then CFO’s responses to sworn interrogatories made clear that a sizable portion of the company’s profitability was driven by price increases on Cuprimine, Syprine, Isuprel, and Nitropress—alone. In the first Quarter of 2016, the ratio of Valeant’s net income from these 4 drugs to total net income was 21.32 percent.⁴¹⁵ Indeed, Mr. Pearson himself was forced to admit this central point at the hearing:

Sen. Tillis: What would you estimate, since the price increase, your profits have been derived, the profits that have been derived from the drugs that we are talking about today, the profit?
Mr. Pearson: I do not have precise numbers, but I would estimate, you know, 10 to 15 percent.⁴¹⁶

E. Indefensible Conduct

Valeant made huge profits by extracting monopoly prices from decades old off-patent drugs. The company’s conduct was so egregious that its executives didn’t even defend Valeant’s actions before the Committee.

Mr. Pearson expressly repudiated the monopoly business model that he helped develop:

[W]e have also made mistakes, including those that bring me here today. In particular, Valeant was too aggressive and I as its leader was also too aggressive in increasing the prices of some of our drugs in our large portfolio of products. In hindsight, I regret

⁴¹³ *Id.* at 73:9–20.

⁴¹⁴ Email from Howard Schiller to J. Michael Pearson, VRX_SCA_00101154 (May 21, 2015).

⁴¹⁵ See Rosiello Interrogatories, at ¶ 8 and attached charts.

⁴¹⁶ April 2016 Hearing, Trans. at. 74:23–75:3 (Apr. 27, 2016).

pursuing transactions where the central premise was based on an increase in price⁴¹⁷

Under questioning from the Committee, Mr. Pearson repeatedly admitted that he had erred in the extreme by pursuing a monopoly pricing strategy:

- “Yes we have been too aggressive on price increases”⁴¹⁸
- “I agree that the price increases were too aggressive. . . .”⁴¹⁹
- **“Sen. Kaine:** . . . Financial Times, October 8, 2015, “Valeant’s business model faces tough questions.” You are quoted. “In an interview with the Financial Times on Tuesday, Mr. Pearson conceded that Valeant’s business model was not fully understood by all investors but insisted the company had ‘nothing to be ashamed of.’” Would that still be your testimony today?
Mr. Pearson: No. In my written testimony and in my oral comments, I think we have been too aggressive—too aggressive on pricing.”⁴²⁰
- “I agree that the price increases were too aggressive. I regret that we took those price increases.”⁴²¹

Valeant’s second largest shareholder at the time of the hearing, William B. Ackman, condemned the behavior even more bluntly at the hearing. Speaking to Syprine and Cuprimine price hikes, Mr. Ackman said: “*Yeah, it is horrible. It is wrong.*”⁴²² In his written testimony, Mr. Ackman said: “Valeant has been appropriately criticized for substantially raising the prices of certain off-patent prescription drugs suddenly and without apparent justification. These issues are worthy of inquiry.”⁴²³

IV. Rodelis Therapeutics

A. Company Background

Rodelis Therapeutics (“Rodelis”) was established in November 2014 and is composed of three different companies: A parent company in Bermuda formed to house intellectual property, an Irish company designed to help with product development, and the U.S.-based Rodelis

⁴¹⁷ *Id.* Trans. at. 48:24–49:6 (testimony of J. Michael Pearson); *see also, id.* at 1 (written testimony of J. Michael Pearson).

⁴¹⁸ *Id.* Trans. at. 61:21–24 (Apr. 27, 2016).
(testimony of J. Michael Pearson)

⁴¹⁹ *Id.* Trans. at. 65:16–17 (Apr. 27, 2016) (testimony of J. Michael Pearson).

⁴²⁰ *Id.* Trans. at. 99:5–15 (Apr. 27, 2016).

⁴²¹ *Id.* Trans. at. 119:23–24 (Apr. 27, 2016) (testimony of J. Michael Pearson).

⁴²² *Id.* Trans. at. 70:13 (Apr. 27, 2016) (testimony of William B. Ackman, Director Valeant) (emphasis added).

⁴²³ *Id.* at 4 (Apr. 27, 2016) (written testimony of William B. Ackman).

Therapeutics.⁴²⁴ Unlike the other three companies investigated by the Committee, Rodelis owned the drug Seromycin on which it took a dramatic price increase for a very short period of time, as it reversed its acquisition within weeks of the price hike.⁴²⁵

B. Seromycin Background

Seromycin (active ingredient: Cycloserine) is used to treat multi-drug resistant tuberculosis (“MDR TB”).⁴²⁶ There are a very small number of cases of MDR TB per year in the U.S.—most experts estimate in the hundreds.⁴²⁷ MDR TB poses a severe public health hazard. It is highly contagious, difficult to treat, life-threatening if left untreated, and preventing its spread is a priority.⁴²⁸ As a result, the CDC is involved in every case diagnosed in the United States.⁴²⁹ The CDC helps coordinate patient care, ensuring that the proper medication is received and that the disease is contained.⁴³⁰ Unlike a typical antibiotic, Seromycin is taken for many months.⁴³¹ While Seromycin is generally tolerated by patients, its use must be closely monitored and other drugs may be needed to blunt side effects.⁴³² Seromycin crosses the blood-brain barrier and in high concentration can have neuropsychiatric effects, which is often addressed through prophylactic administration of Vitamin B6, as well as frequent post-prescription medical monitoring and care.⁴³³

Seromycin is an old drug—Eli Lilly, Co. (“Lilly”), brought it to market in 1964⁴³⁴, and the Committee has not identified any material changes in its composition since that time.

C. The Acquisition of Seromycin

The rights to produce Seromycin were transferred by Lilly in 2007 to the Chao Center (“Chao”), which operates under Purdue University.⁴³⁵ Chao was designed as a small drug manufacturing center that would also be used to educate pharmacy students.⁴³⁶ The only drug

⁴²⁴ Committee Staff Briefing with Counsel for Rodelis (Dec. 1, 2015) (“Rodelis Briefing”).

⁴²⁵ The company has represented that no patient paid this price.

⁴²⁶ Seromycin also can be used to treat urinary tract infections, and has shown some experimental benefit in treating psychiatric conditions. *See, infra*, at note 430.

⁴²⁷ Rodelis Therapeutics, Company Overview, RTI-USSSCA-00000321, at RTI-USSSCA-00000333 (Aug. 2015).

⁴²⁸ *Id.* at RTI-USSSCA-00000334.

⁴²⁹ *See generally*, The White House, *National Action Plan for Combating Multidrug-Resistant Tuberculosis* (Dec. 2015).

⁴³⁰ *Id.*

⁴³¹ *See* Email from Srihari Vedartham to Joseph Stowell, RTI-USSSCA-00002496, at RTI-USSSCA-00002496 (Apr. 21, 2015) (dosing of 18–24 months for MDR TB, 5–10 days for urinary tract infections).

⁴³² Rodelis Briefing; *see also*, Seromycin FDA Label (13845-1200-3)..

⁴³³ *Id.*

⁴³⁴ *See* Rodelis Therapeutics, Company Overview, RTI-USSSCA-00000321, at RT-USSSCA-00000333 (Aug. 2015).

⁴³⁵ Under the terms of the transfer, Lilly retained the IP but granted a perpetual license to the Chao Center for the U.S. and Canada. *See* Letter from Andrew Dahlman (Eli Lilly & Co.) to Brian Edelman, RTI-USSSCA-00000216 (Aug. 17, 2015).

⁴³⁶ Committee Staff Interview with Daniel J. Hesler, CEO Perdue Foundation (Nov. 18, 2015) (“November Hesler Interview”).

produced at Chao is Seromycin, and production runs occur once a year and take about one to two weeks.⁴³⁷ The facility is required to comply with all applicable FDA regulations.⁴³⁸

Due to high sunk manufacturing costs and the small market size of Seromycin, Chao suffered a series of losses, which it estimated to be \$10 million, prior to the sale of Seromycin to Rodelis on August 19, 2015.⁴³⁹ At that time, the drug was priced at about \$500 for 30 capsules, generating about \$1 million a year in revenue for Chao, against \$2 million in annual expenses.⁴⁴⁰

In the period leading up to the Rodelis transaction, Chao's board determined that while it could not cease making Seromycin due to the acute medical need it served, management should seek to divest the asset while ensuring its continued availability.⁴⁴¹ While Chao did not issue a request for proposal, it made it known in pharmaceutical circles that it was looking to divest Seromycin.⁴⁴²

Rodelis contacted Chao on April 3, 2015, to initiate a conversation about purchasing Seromycin.⁴⁴³ Negotiations took place over the next five months and involved discussions of the potential for price increases, securing the entire supply of the API, and ways to limit exposure to government payers.⁴⁴⁴ Rodelis and Chao completed the sale of Seromycin on August 19, 2015.⁴⁴⁵

The Rodelis principals who primarily handled contact negotiations were Mr. Michael Goldstein ("Goldstein") a partner at Avego, Mr. Bala Venkatarmann ("Venkatarmann"), another partner at Avego, and Mr. Srihari Vedartham ("Vedartham"), Rodelis' VP of Business Development. Negotiations for Chao were handled primarily by its CFO, Mr. Brian Edelman ("Edelman"). Under the terms of the deal, Chao transferred its license for Seromycin in the U.S. and Canada to Rodelis.⁴⁴⁶ In return, Rodelis made a cash payment of \$4.125 million, acquired all existing product and API for another \$1.1 million, and entered into a long term supply

⁴³⁷ The Center's manufacturing capabilities are dormant for most of the year. Despite this, the Center incurs substantial costs throughout the year. It must pay the salary of three fulltime employees, pay full GUDFA fees of approximately \$250,000 a year, rent space, and service the infrastructure required to comply with FDA manufacturing regulations. Hesler Interview. The Chao Center pays the same FDA fees as much larger facilities. Requests for a discount to reflect (at least in part) the Chao Center's educational mission were denied). *Id.*

⁴³⁸ *Id.*

⁴³⁹ *Id.*

⁴⁴⁰ Committee Staff Interview with Daniel J. Hesler (Jan. 15, 2016) ("January Hesler Interview"); November Hesler Interview.

⁴⁴¹ January Hesler Interview; November Hesler Interview.

⁴⁴² November Hesler Interview.

⁴⁴³ See Email from Srihari Vedartham to Joseph Stowell, RTI-USSSCA-00002461 (Apr. 3, 2015).

⁴⁴⁴ See, e.g., generally, Email from Brian Edelman (Chao Center), RTI-USSSCA-00001210 (May 15, 2015); Email from Joseph Stowell to Srihari Vedartham, RTI-USSSCA-00002575 (July 20, 2015); Email from Brian Jennette to Michael Goldstein, RTI-USSSCA-00007974 (July 27, 2015); Email from Joanna Young to Michael Goldstein, RTI-USSSCA-00007244 (July 17, 2015).

⁴⁴⁵ See Rodelis Therapeutics, Company Overview, RTI-USSSCA-00000321, at RTI-USSSCA-00000338 (Aug. 2015).

⁴⁴⁶ See Rodelis Therapeutics, Company Overview, RTI-USSSCA-00000321, at RTI-USSSCA-00000338 (Aug. 2015).

contract (with minimum supply provisions at fixed pricing).⁴⁴⁷ That *same day* Rodelis raised the price of Seromycin 2,060 percent, from \$500 for 30 capsules to \$10,800 for 30 capsules.⁴⁴⁸

1. Monopoly Pricing Power and Substantial Profits

Rodelis' internal documents reveal that the company viewed this transaction as a way to generate substantial profits by taking a massive price increase. The first financial model Rodelis (dated June 22, 2015) created after signing the operative Indication of Interest⁴⁴⁹ provided for a more than tenfold price increase, and after being validated for "OPEX and GTN," led to a truly incredible projected IRR of over 270 percent on the transaction over the model horizon.⁴⁵⁰ A more-refined internal model, created on July 14, 2015, assumes almost the exact price increase eventually taken—\$350 a capsule, a 21 fold price increase (the actual increase was \$360⁴⁵¹).⁴⁵² This spreadsheet projects that the price increase will lead to a profitable transaction—a 2.67 fold return on the initial investment.⁴⁵³ A presentation dated August 26, 2015 (seven days after the transaction closed), and apparently intended for investors, projected similarly massive profits driven by an impressive gross margin, as shown in Figure 9.

⁴⁴⁷ See generally, Purchase and Sale Agreement, RTI-USSSCA-00000075 (Aug 19, 2015).

⁴⁴⁸ See Rodelis Therapeutics Notice of Price Increase, AVEGO-000001177, at AVEGO-000001177 (Aug. 19, 2015).

⁴⁴⁹ The operative Indication of Interest was dated June 3, 2015. See Indication of Interest, RTI-USSSCA-00000001 (Jun. 3, 2015).

⁴⁵⁰ See Email from Srihari Vedartham to Bala Venkataraman, et al., AVEGO-00000345 (June 22, 2015), and accompanying attachment, Excel Spreadsheet, AVEGO-00000346 (undated).

⁴⁵¹ See Rodelis Notification of Price Increase, AVEGO-00001177 (Aug. 19, 2015).

⁴⁵² See Excel Spreadsheet, RTI-USSSCA-00000262 (July 14, 2015).

⁴⁵³ *Id.*

Figure 9. Rodelis Projected Financial Metrics.⁴⁵⁴

(\$ in 000's)						
Rodelis Therapeutics						
Seromycin Acquisition	Stub* 4 Mos. 2015P	2016P	2017P	2018P	2019P	2020P
U.S. Capsules Shipped	20,088	51,322	38,492	28,869	21,652	16,239
% Change	NA	NA	-25%	-25%	-25%	-25%
Net Sales	\$1,369	\$12,781	\$10,739	\$8,376	\$6,533	\$5,096
COGS	213	933	933	376	282	290
Gross Profit	\$1,156	\$11,848	\$9,805	\$8,001	\$6,252	\$4,806
% Margin	84%	93%	91%	96%	96%	94%
SG&A	1,605	2,997	2,903	2,847	2,540	2,528
EBITDA	(\$449)	\$8,851	\$6,902	\$5,154	\$3,712	\$2,278
Less: Cash Taxes	(13)	266	207	155	111	68
Cash Net Income	(\$436)	\$8,585	\$6,695	\$4,999	\$3,600	\$2,210

* Assumes Seromycin acquisition closes 8/31/15.
Note: Forecast assumes no contribution from ex-U.S. sales.

Communications between the parties prior to closing indicate that both parties viewed the ability to substantially raise prices on Seromycin as a key transaction selling point. For example, after Chao rejected a term sheet, and Rodelis counteroffered, Chao's CFO sent a further counterproposal and noted "with the significant value that exists in the one simple, powerful lever of pricing for Seromycin, we believe ours is a fair, final proposal. We have not experienced any market responses from our past price increases and are currently preparing to implement a series of price increases."⁴⁵⁵

Rodelis' internal documents from before and after the transaction show how the company implemented many aspects of the business model identified by the Committee in order to generate extreme profits. Rodelis emphasized that its approach was to implement "[o]rphan drug strategies "(i.e., acquire small market drugs that are the only treatment for serious illness and then raise prices).⁴⁵⁶

⁴⁵⁴ Rodelis Therapeutics, Company Overview, RTI-USSSCA-00000321, at RTI-USSSCA-00000340 (Aug. 2015). Goldstein told the Committee that he did not recall the presentation's audience, but it was possible that the audience was both investors and other financial institutions. Committee Staff Interview with Michael Goldstein (Nov. 16, 2016). Goldstein did state that this slide presented key numbers that would be of importance to investors. *Id.*

⁴⁵⁵ Email from Brian Edelman to Srihari Verdartham, RTI-USSSCA-00001210, at RTI-USSSCA-00001210 (May 15, 2015).

⁴⁵⁶ Rodelis Therapeutics, Company Overview, AVEGO-00000244, at AVEGO-00000253 (Apr. 2015). Of course, treating orphan diseases is a commendable endeavor. But the Committee's investigation revealed that the phrase "orphan drug strategy" is code for a strategy of using a de facto monopoly to extract massive profits.

2. Sole-Source & Gold Standard

Internal Rodelis documents recognized that Seromycin was valuable and ripe for a monopoly pricing strategy because it provided a unique option for treating a potentially deadly condition. In July 2015, Rodelis employees researching the business case for the acquisition of Seromycin highlighted that: “Seromycin is the *only* product that has the ability to treat both pulmonary and extra pulmonary infection.”⁴⁵⁷ Similarly, when looking at an alternate use of Seromycin—treating antibiotic resistant urinary tract infections—Mr. Vedartham highlighted the fact that: “We looked at some of the published lit around the UTI, especially drug being used in infections resistant to trimethoprim and third generation cephalosporins. *This would make it last line therapy to certain extent.*”⁴⁵⁸

A post-transaction presentation, which appears to have been designed at least in part for investors, continues the theme. It begins by highlighting the seriousness of MDR TB and noting that “Seromycin® is one of the last-line MDR therapies.”⁴⁵⁹ It then goes on to devote an entire page to “Multiple-Drug Resistant TB (MDR)” which highlights the “severe” nature of TB and specifically notes that “Seromycin® is the only drug approved for MDR that treats both pulmonary and extrapulmonary TB.”⁴⁶⁰ The presentation concludes with the following bullet points:

- “Treats a medically necessary condition, multiple-drug resistant TB.”⁴⁶¹
- “Last line treatment when first line medications have failed.”⁴⁶²

3. Small Market

Rodelis’ external materials repeatedly stressed that Rodelis was focused on small market (*i.e.*, orphan drugs) and that Seromycin was such an orphan drug. In a presentation dated April 2015 that appears to have been sent to investors, Rodelis explained that the name Rodelis was an acronym for “The Rodelis Values,” the first of which was “r” reflecting “rare disease” as Rodelis’ “focus,” and the last of which was “s” reflecting Rodelis’ focus on drugs treating “Small patient populations. . . .”⁴⁶³ This theme was echoed in Rodelis’ post-transaction

⁴⁵⁷ Email from Bala Venkataraman to Srihari Vedartham, AVEGO-00000773 (July 16, 2015) (emphasis added).

⁴⁵⁸ Email from Srihari Vedartham to Joanna Young, RTI-USSSCA-00007891, at RTI-USSSCA-00007891 (July 23, 2015) (emphasis added).

⁴⁵⁹ See Rodelis Therapeutics, Company Overview, RTI-USSSCA-00000321, at RTI-USSSCA-00000333 (Aug. 2015); *supra*, note 78.

⁴⁶⁰ *Id.* at RTI-USSSCA-00000334.

⁴⁶¹ *Id.* at RTI-USSSCA-00000341.

⁴⁶² *Id.* Rodelis also contemplated acquiring another drug, at this time and in the same presentation touted that the drug was “last-line,” “life changing” therapy for an extraordinarily severe neurological condition. *Id.* at RTI-USSSCA-00000343 and RTI-USSSCA-00000352.

⁴⁶³ Rodelis Therapeutics, Company Overview, AVEGO-00000244, at AVEGO-00000247 (Apr. 2015). The Committee credits Rodelis’ intent to provide the capital that was necessary to ensure the continued production of an essential drug. See, *infra*, at 83–84. But that cannot wipe out the fact that when that intent is intertwined with an intent to use a *de facto* monopoly to price gouge, the end result is fundamentally wrong.

presentation (which appears to have been presented to investors) which stated that “Rodelis Therapeutics is a rapidly growing specialty pharmaceutical company focused on orphan diseases and conditions with high unmet medical need.”⁴⁶⁴ This presentation also touted the fact that Seromycin was a “[s]mall volume, ultra-orphan product treating <200 patients annually.”⁴⁶⁵

4. Delaying Generic Entry

Rodelis expected no generic entry, despite the sizable price increase it had taken on Seromycin.⁴⁶⁶ Documents reviewed by the Committee show that Rodelis intended to take a variety of steps to deter generic entry. In one example, Rodelis actively sought to enter into an exclusivity deal with the only known supplier of Cyclosporine, the API in Seromycin.⁴⁶⁷ Rodelis’ presentations, both leading up to the deal and after, repeatedly emphasized Rodelis’ expertise in this regard:

- “Key Product Characteristics. . . . Strong IP, market exclusivities and/or manufacturing barriers.”⁴⁶⁸
- “Multiple regulatory strategies.”⁴⁶⁹
- “Seromycin® Investment Summary. . . . Several defensive mechanisms and barriers of entry for generic competition.”⁴⁷⁰

5. Price Gouging

Setting aside the sheer magnitude of the price increase, which is itself suggestive of price gouging, Rodelis’ actions surrounding the price increase reveal a motive to reap monopoly profit. Rodelis asserted to investors, the media, and in its presentation to the Committee, that the price increase would be mitigated by robust patient assistance programs and improved access programs.⁴⁷¹ But internal Rodelis documents and Rodelis’ actions paint a different picture. Rodelis took its price increase the same day it acquired the drug, but delayed implementation of its promised patient assistance and access programs. Rodelis publicly acknowledged that price

⁴⁶⁴ Rodelis Therapeutics, Company Overview, RTI-USSSCA-00000321, at RTI-USSSCA-00000324 (Aug. 2015).

⁴⁶⁵ *Id.* at RTI-USSSCA-00000341. In another transaction contemplated by Rodelis, they highlighted that the drug was for “[u]ltra-orphan use.” *Id.* at RIT-USSSCA-00000343.

⁴⁶⁶ See Email from Srihari Vedartham to Bala Venkataraman, et al., AVEGO-00000345 (June 22, 2015), and accompanying attachment, Excel Spreadsheet, AVEGO-00000346 (undated).

⁴⁶⁷ See Email from Bala Venkataraman to Srihari Vendartham, AVEGO-00000881 (July 30, 2015). See also, Email from Srihari Vendartham to Bala Venkataraman, AVEGO-00000805, at AVEGO-00000805–AVEGO-00000806 (July 22, 2015) (discussing that there was only one known supplier of Seromycin API).

⁴⁶⁸ Rodelis Therapeutics, Company Overview, AVEGO-00000244, at AVEGO-00000250 (Apr. 2015).

⁴⁶⁹ *Id.* at AVEGO-00000253.

⁴⁷⁰ Rodelis Therapeutics, Company Overview, RTI-USSSCA-00000321, at RTI-USSSCA-00000341 (Aug. 2015). In another potential transaction discussed in the presentation, Rodelis intended to deter generic competition with a decades old drug by joining with the manufacturer of the pump that administers the drug to “not allow access to any analytical data or [REDACTED] pumps to allow for potential future competitors.” *Id.* at RTI-USSSCA-00000346; see also *id.* at RTI-USSSCA-00000352 (“Exclusive arrangement with [REDACTED] whereby they will not supply the pump or analytical data to third parties, limiting generic risk.”).

⁴⁷¹ Committee Staff Interview with Counsel for Rodelis (Dec. 1, 2015).

increases could hurt patients, and touted that it would implement programs to address this concern but failed to put such programs in place.

The 340B program provides low cost drugs to the most vulnerable in our population, and numerous pharmaceutical companies willingly participate in this program.⁴⁷² But Rodelis appears to have made efforts to limit its participation in the 340B program. In July, Avego commissioned a well-known consulting firm, Avalere, to among other things, “[a]nalyze 340B chargeback and wholesaler sales data . . . to assess the 340B exposure, and assess the implications of switching the drug distribution to specialty pharmacy providers (SPPs): requirements, feasibility, 340B impact, patient access, and PR considerations.”⁴⁷³ This work seems to have been done to explore limiting the amount of Seromycin prescriptions filled under the 340B program in favor of more profitable channels. The presentation spoke of implementing restricted distribution because it had: “[p]otentially less 340B exposure (though more and more SPPs are becoming 340B contract pharmacies).”⁴⁷⁴ It then makes the following points about “limiting 340B exposure”:⁴⁷⁵

- “SPP distribution network might have a variable impact on 340B discounts depending on the entity-specific characteristics.”⁴⁷⁶
- “SPPs might place additional administrative burden for 340B entities which may discourage them from purchasing the product.”⁴⁷⁷
- “Limiting distribution to select SPPs also limits point so negotiation for sub-ceiling pricing by 340B entities (*e.g.*, the Prime Vendor Program.)”⁴⁷⁸

Shortly after receiving this presentation, a Rodelis employee wrote in apparent answer to a question from Mr. Goldstein:

We cannot close off direct sales to 340b institutions. If a 340b institution wants to buy the drug (via an SP channel or not), we have to let them and give them PHS pricing. Also, most SP’s are now selling 340b direct because the gov’t is pushing them to do so.⁴⁷⁹

⁴⁷² See, *infra*, at 130.

⁴⁷³ Email from Joanna Young (Avalere) to Srihari Vedartham and Michael Goldstein, RTI-USSSCA-00007891, at RTI-USSSCA-00007893 (July 24, 2015).

⁴⁷⁴ Email from Joanna Young to Srihari Vedartham & Michael Goldstein, RTI-USSSCA-00007891 (July 24, 2015), and accompanying attachment, Avalere, Considerations Around Changes to the Drug Distribution Strategy, RTI-USSSCA-00007895, at RTI-USSSCA-00007899 (July, 2015).

⁴⁷⁵ *Id.* at RTI-USSSCA-00007902.

⁴⁷⁶ *Id.*

⁴⁷⁷ *Id.*

⁴⁷⁸ *Id.*

⁴⁷⁹ Email from Michael Goldstein to Srihari Vedartham, RTI-USSSCA-00007974 (July 27, 2015). Mr. Goldstein told the Committee that this email merely represented an attempt to derive all sales through specialty pharmacies—not to block all 340B sales. While we have no reasons to question the veracity of this statement, it seems a distinction without a difference. The previous documents evidence an intent to switch to specialty distribution to, at a minimum, drive down 340B. While the product was never placed into restricted distribution, there were active post-acquisition discussions with vendors to do so. See, *e.g.*, Email from Brian Jennette to Melinda Koehler, RTI-USSSCA-00007998 (Aug. 20, 2015).

Rodelis repeatedly emphasized in its internal presentations that it intended to provide patient assistance to “ensure all patients will have access to critical therapies.”⁴⁸⁰ This included assistance via a “[r]eimbursement hub with un-capped copay assistance,”⁴⁸¹ and an “[o]ppportunity to provide high-touch service to patients and improve access through Patient Assistance Program (PAP) and copay assistance.”⁴⁸² In one example, Rodelis’ outside media consultant advised responding to criticism of Rodelis’ Seromycin price increase by pushing the message that Rodelis “is committed to ensuring the reliable availability” of Seromycin.⁴⁸³ In draft messaging documents, the same consultant advised stating that “Rodelis is committed to ensuring reliable availability of this important medicine for the treatment of patients with MDR-TB. This company will continue to work with all relevant parties, (payers, hospital, health departments, physicians and patients) to ensure the availability of cycloserine.”⁴⁸⁴ And on September 15, Mr. Spencer told *The New York Times* that Rodelis “was committed to ensuring access to treatment and as such Rodelis is . . . supporting a patient assistance program whereby uninsured patients can apply to access the medication for free.”⁴⁸⁵ *The New York Times* wrote, Mr. Spencer said Rodelis “provided the drug free to certain needy patients.”⁴⁸⁶

Rodelis implemented the price increase so quickly that the drug’s wholesaler, ICS, was unable to replace Chao’s contact information with Rodelis’ in time to field inquiries about the price increase. As Chao explained it contemporaneously, “[u]nfortunately they didn’t wait until they had ICS IT create an email address for themselves, [to raise the price] so [Chao] is still loosely associated with the price increase. Also, I’ve asked Brian J to give me the phone number to which they want us to forward calls because I know calls will be coming in sooner than later.”⁴⁸⁷

Additionally, even though Rodelis attempted to justify the price increase by saying that it would invest profits in programs designed to assist patients taking Seromycin, it implemented the price increase despite the fact that Rodelis was still in the nascent stages of planning for these enhanced patient programs. The company had not yet even met with its preferred potential provider to start the process of ironing out program details.⁴⁸⁸ Despite Rodelis’ repeated public

⁴⁸⁰ Email from Virinder Nohria to Bala Vekataraman AVEGO-00000243 (Jun. 3, 2015), and accompanying attachment, Rodelis Therapeutics, Company Overview, AVEGO-00000244, at AVEGO-00000247 (Apr. 2015).

⁴⁸¹ See, e.g., Rodelis Therapeutics, Company Overview, RTI-USSSCA-00000321, at RTI-USSSCA-00000328 (Aug. 2015); Rodelis Therapeutics, Company Overview, AVEGO-00000244, at AVEGO-00000250 (Apr. 2015).

⁴⁸² Rodelis Therapeutics, Company Overview, RTI-USSSCA-00000321, at RTI-USSSCA-00000341 (Aug. 2015);

⁴⁸³ Centrion, Medial Training Session, Rodelis Therapeutics, AVEGO-00002672, at AVEGO-00002683 (Aug. 21, 2015)

⁴⁸⁴ Email from Melinda Koehler to Verender Nohria, VN-00000012, (Sept. 4, 2015), and accompanying attachment, DRAFT Seromycin® (Cycloserine) Q&A, VN-00000016, at VN-00000018 (Aug. 21, 2015).

⁴⁸⁵ Email from Scott Spencer to Andrew Pollack, RTI-USSSCA-00005071, at RTI-USSSCA_00005072 (Sept. 15, 2015).

⁴⁸⁶ Andrew Pollack, *Drug Goes From \$13.50 a Tablet to \$750, Overnight*, New York Times (Sept. 20, 2015).

⁴⁸⁷ Email from Srihari Vendartham to Melinda Koehler and Brian Jennette, AVEGO-00001176, at AVEGO-00001176 (Aug. 21, 2015).

⁴⁸⁸ See Email from Brian Jennette to Melinda Koehler, RTI-USSSCA-00007998, at RTI-USSSCA-00007998 (Aug. 20, 2015). To be sure, Rodelis was displeased that the meeting did not occur sooner. *Id.* But they did not delay the price increase.

claim that no one would go without the drug, the first documented efforts to set up a patient assistance program appear to have occurred in September—weeks *after* the price increase was put in place.⁴⁸⁹ Indeed, as late as September 14, Mr. Goldstein was emailing bankers stating “[w]e *plan* to implement a PAP Free drug program for cycloserine.”⁴⁹⁰ And even the planned PAP program only applied to patients without insurance—those with insurance would apparently have to wait for weeks after the price increase for planning on co-pay assistance to begin.⁴⁹¹ Rodelis, on the other hand, had time not only to increase the price of Seromycin, but also to receive a comprehensive media training presentation on August 21, 2015, focused on defending the price increase by touting the very PAPs that do not appear to have even existed at that time.⁴⁹²

6. Rodelis Reacts to Intense Public Pressure

Subsequent to the price increase taking effect, Rodelis faced fire from virtually every corner.⁴⁹³ On September 18, 2015, in the face of mounting pressure, Chao requested that Rodelis reverse the transaction.⁴⁹⁴ Rodelis agreed to do so within about 72 hours⁴⁹⁵ and the transaction was officially reversed on September 21. The terms of the reversal restored both parties to the pre-transaction status quo.⁴⁹⁶

It bears noting that while Rodelis engaged in price gouging, Chao could not continue to make Seromycin at its pre-transaction price point due to the economics of production, and some sort of price increase was necessary to support the ongoing production of this vital drug.⁴⁹⁷ Post transaction, Chao doubled the price of Seromycin to \$1,050 for 30 capsules (110 percent above the pre-transaction price).⁴⁹⁸ At this price, Chao informed the Committee that it will just break

⁴⁸⁹ See Email from Melissa Koehler to Autumn (NeedyMeds), RTI-USSSCA-00006920 (Sept. 5, 2015). To be sure, there were earlier theoretical discussions in this vein. See Letter from Michael D. Bopp, Esq. to the Hon. Susan M. Collins & the Hon. Claire McCaskill, Attachment, at 2 (Dec. 7, 2015), SAOL-00000307 at SAOL-00000308.

⁴⁹⁰ Email Michael Goldstein to James Jonathan, RTI-USSSCA-00007201, at RTI-USSSCA-00007201 (Sept. 14, 2015) (emphasis added).

⁴⁹¹ See Email from Richard Sagall to Melinda Koehler and Attachment, (Sept. 15, 2015). RTI-USSSCA-00007097, *and accompanying attachment*, Rodelis Therapeutics Patient Assistance Program for Seromycin® (Cycloserine), RTI-USSSCA-00007102, at RTI-USSSCA-00007103 (Sept. 15, 2015). To be sure, Rodelis did attempt to provide a stop-gap “Compassionate Use” program. But this stop-gap program was not deployed until September 9, 2015. See Email from Zachariah Humleker, Esq. to Samuel Everett Dewey, Esq. (Nov. 29, 2016). And this occurred only after Rodelis received inquiries as to when their putative patient assistance program would be operation. See Email from Michael D. Bopp to Samuel Everett Dewey, Esq. (Nov. 25, 2016). Rodelis was unable to produce any documents describing the details of the program which appears to have only provided 30 days of free medication. See Email from Michael D. Bopp to Samuel Everett Dewey, Esq. (Nov. 25, 2016).

⁴⁹² See Email from Melinda Koehler to Shamm Astute, AVEGO-00002670, at AVEGO-00002670 (Sept. 3, 2015).

⁴⁹³ See, .e.g., Andrew Pollack, *Drug Goes From \$13.50 a Tablet to \$750, Overnight*, N.Y. Times (Sept. 20, 2015).

⁴⁹⁴ November Hesler Interview; Email from Daniel J. Hesler to Brian Edelman, AVEGO-00001141 (Sept. 18, 2015).

⁴⁹⁵ November Hesler Interview (Nov. 18, 2015)

⁴⁹⁶ See Addendum to Purchase and Sale Agreement, RTI-USSSCA-00000223, at RTI-USSSCA-00000223 (Sept. 21, 2015).

⁴⁹⁷ January Hasler Interview (Jan. 15, 2016); November Hasler Interview (Nov. 18, 2015).

⁴⁹⁸ *Id.*

even⁴⁹⁹ and will not be able to retire its \$10 million in outstanding debt, or underwrite needed patient support.⁵⁰⁰ Given the economics of this small market drug, Chao is uncertain about its continued ability to produce Seromycin, and is concerned that the drug will eventually cease being made in the United States.⁵⁰¹

⁴⁹⁹ *Id.*

⁵⁰⁰ *Id.*

⁵⁰¹ January Hasler Interview.

CHAPTER 4. ROLE OF INVESTORS

This section examines how each of the four companies in this investigation had activist investors pushing them to adopt the strategies embodied in the business model as well as the investor-like approach of senior management at these companies.

Investors play an important role in the modern company, not only in providing financing, but in functioning as a check on corporate Boards and the management of a company. Activist investors, in fact, can be a source of good and help to prevent or deter corporate excesses. This dynamic, however, appears to have been turned on its head in the case of the companies investigated by the Committee. Evidence suggests that in these cases activist investors were part and parcel of the problem, pressing the companies to adopt and implement the business model identified by the Committee. Activist investors in these cases may have worsened and verified decisions made by executives.

The companies investigated by the Committee were also often headed by senior management who lacked pharmaceutical backgrounds and hailed from hedge funds. This is relatively unusual in the realm of traditional pharmaceutical companies and helps explain why the companies investigated by the Committee may have at times acted more like hedge funds than traditional pharmaceutical companies.

I. Retrophin, Inc.

Retrophin, Inc. was founded in February 2011 by Mr. Shkreli, who subsequently founded Turing after he was ousted from Retrophin. As discussed in Chapter 3, internal Retrophin documents suggest a returns-driven strategy motivating Retrophin's acquisitions and price increases on drugs such as Thiola. Emails between Dan Wichman of Broadfin Capital and Mr. Shkreli show that Mr. Wichman was intimately involved in advising on the company's strategy, down to cautioning Mr. Shkreli about his social media presence and advising him against tweeting about the FDA.⁵⁰²

In one example of investor involvement, Mr. Shkreli and Mr. Wichman discuss Retrophin's price-gouging strategy and shift from waxing philosophical about the blindness of traditional pharmaceutical companies to price-gouging opportunities to discussing specific goals

⁵⁰² See, e.g., Email from Dan Wichman to Martin Shkreli, SSICA_THIOL_037842, at SSICA_THIOL_037842 (May 5, 2014); Email from Dan Wichman to Martin Shkreli, SSICA_THIOL_037848, at SSICA_THIOL_037848 (May 5, 2014); Email from Dan Wichman to Martin Shkreli, SSICA_THIOL_037863, at SSICA_THIOL_037863 (May 5, 2014); and Email from Dan Wichman to Martin Shkreli, SSICA_THIOL_037871, at SSICA_THIOL_037871 (May 5, 2014).

for the Thiola deal to blithely acknowledging that they should not be in such frequent contact about the company's plans:⁵⁰³

[5/1/2014 2:41 p.m. Shkreli to Wichman] "*We are doing the entire deal at \$190m. You twisted my arm!*"

[5/1/2014 2:45 p.m. Wichman to Shkreli] "They've agreed to this? All parties? If so, that is great news, and we'd be very excited. *Happy to pick up \$10mln in pre-paid royalties to make those clowns happy. The npv is a no-brainer.*"

[5/1/2014 2:57 p.m. Shkreli to Wichman] "Yes. It should be a done deal. Never say never though."

[5/2/2014 5:41 p.m. Wichman to Shkreli] "Any word on the r+d guy yet?"

[5/3/2014 7:57 p.m. Shkreli to Wichman] "*I have to be careful with giving you minute by minute updates on the company ☺*"

[5/3/2014 8:13 a.m. Wichman to Shkreli] "*Yes fair enough—once this deal closes I'll go back to being less of a pain in the a\$\$. . . Assuming this looks like a done deal this week (knock on wood), I'd love to discuss a little of how you'll convey it to the Street—I'm sure you've spent many hours thinking about that. . . . Then I'll go back to leaving you alone and not harassing you semi-hourly—let you do the hard work in creating value.*"

[5/3/2014 8:14 a.m. Shkreli to Wichman, referring to Thiola for the first time] "What if I told you we might announce two deals at once?"

[5/3/2014 8:19 a.m. Wichman to Shkreli] "I'd say, I'll be happy with the one I know about, but I'm always open to more as long as you guys have the personnel and time and expertise to handle it all."

[5/3/2014 8:23 a.m. Shkreli to Wichman] "We'd pay \$1m to acquire a drug called Thiola, which is the only treatment for a rare disease called cystinuria *The drug does \$1.2m in sales. It is woefully underpriced and would not stop selling at orphan prices. With new pricing we estimate sales of \$20 to \$40 million. Almost 95% EBITDA margins at those prices. Would be an annuity for some time.*"

[5/3/2014 8:41 a.m. Wichman to Shkreli] "Interesting—sounds like a no-lose, to put it mildly. Don't have to run a model on that one this weekend to give you my opinion.

Funny that these small companies still haven't realized you can raise price aggressively and nobody gets too upset? Obviously depends on the product—but I figure this dynamic may not last forever, you need to maximize opportunities while you can. . . . It's not like people are giving companies gold stars for charging slightly lower prices ("thanks guys for charging 500 an rx not 800")—in that land the generics aren't your competition and don't even try. . . .

Anyway, it's different in orphan land, and probably more sustainable, but *seems like at this point these little guys would get the idea that they could push things a*

⁵⁰³ Email from Dan Wichman to Martin Shkreli, SSCA_THIOL_037832, at SSCA_THIOL_037832 - SSCA_THIOL_037836 (May 3, 2014) (emphases added). This email discusses not only the Thiola transaction, but other potential transactions as well. Mr. Wichman indicated in his hearing testimony that at this time, he was primarily focused on another transaction that was for a large volume drug for which Broadfin was providing financing. Mr. Wichman stated that Broadfin's investment thesis for Retrophin was not related to the business model and the sentiments expressed in these emails.

bit. How can they ever make money with that model? Bottom line is I won't get too excited but it sounds very intriguing."

[5/3/2014 8:47 a.m. Shkreli to Wichman] *"The drug companies are afraid. Small ones, big ones, etc. Big price increases are horrifying because most executives overestimate changes in demand. It comes mostly from pharma's history as quasi-consumer products. The next generation of pharma guys (or the smart ones) understand the inelasticity of certain products. The insurers really don't care. They just pass it through and focus on managing care for physician payments and blockbusters."*

[5/3/2014 1:04 p.m. Wichman to Shkreli] *"I hear you on the pharma mentality—it's ironic how it took two companies—jazz and hzn—on the brink of insolvency to decide they should aggressively play the price card. . . . And qcor is obviously a poster-child—for the heat and bad PR they took, didn't work out so badly in the end, did it? Not every deal and every product will work out like these, but for smart managements, that are resourceful and opportunistic, these are exciting times."*

In another email chain, referenced earlier, Mr. Wichman discusses with Mr. Shkreli the possibility of Congress acting to prevent closed-distribution schemes from keeping generics out of the market, as well as the implications of such an action for their business model.⁵⁰⁴

These emails illustrate how much influence activist investors may have in pharmaceutical companies that are acting more like hedge funds. The emails also revealed an instance in which Mr. Shkreli states that Retrophin's biggest shareholder was dictating the timing of a press release regarding a deal and that he had to defer to that shareholder's timing preference because "their wish is my command."⁵⁰⁵

Investors such as Mr. Wichman claim that they are not activist investors because they are not buying shares in order to take control of the company.⁵⁰⁶ This, however, misses the point. If these investors have influence in the functioning of the company and if, along with the senior management of these pharmaceutical companies, they are actively involved in pushing the business model identified by the Committee, at the expense of patients and the health care system, then there is little practical difference.

II. Turing Pharmaceuticals, LLC

Mr. Shkreli founded Turing Pharmaceuticals, LLC after he was ousted from Retrophin. Turing's largest shareholder and former CEO is Mr. Shkreli. The company launched on February 24, 2015, and stated that its focus was "address unmet medical needs."⁵⁰⁷ The

⁵⁰⁴ See Email from Dan Wichman to Martin Shkreli, SSICA_THIOL_038413, at SSICA_THIOL_038413-14.

⁵⁰⁵ Email from Martin Shkreli to Jim Self, SSICA_THIOL_007874, at SSICA_THIOL_007874 (May 30, 2014) (stating "Monday is the Jefferies conference—so we ordinarily would never front-run something like this, but we simply couldn't put this news out on Monday according to our biggest shareholder (their wish is my command)").

⁵⁰⁶ See March 2016 Hearing, at 2 (written testimony of Dan Wichman).

⁵⁰⁷ Turing Pharmaceuticals Press Release (Feb. 24, 2015), *found at*, <http://www.turingpharma.com/media/press-release?headline=launch-of-turing-pharmaceuticals-announced-%25e2%2580%2593-new-drug-company-to-focus-on-treating-unmet-medical-needs-across-broad-therapeutic-areas> (last visited Dec. 7, 2015).

company purchased several drug products in various stages of regulatory approval from Retrophin. (These drugs are still in the approval phase.)⁵⁰⁸

Internal investor presentations indicate that Mr. Shkreli planned to execute an investor-driven strategy at Turing, similar to what he did at Retrophin. These presentations highlighted that Daraprim fit the business model identified by the Committee and that a huge price increase would lead to massive returns.⁵⁰⁹ Although Mr. Wichman considered investing in Turing and emails show that significant and sensitive information was provided to Mr. Wichman about the Daraprim acquisition (prior to its closing) to encourage him to invest, he ultimately decided not to do so, citing concerns about Mr. Shkreli's personality and viability as a successful CEO.⁵¹⁰

Shortly after founding Turing, Mr. Shkreli made its first large acquisition—Daraprim. Daraprim had precisely the characteristics that Mr. Shkreli and Mr. Wichman discussed in detail over email at Retrophin.⁵¹¹

In founding Turing, Mr. Shkreli brought over his inner circle from Retrophin, including Mr. Tilles, Mr. Urrutia, Mr. Crutcher, and Mr. Smith, who all focused on courting investors. Despite Mr. Shkreli's resignation from the company, these members of his inner circle remained in positions of control at Turing. Most notably, Mr. Tilles was installed as the Chairman of the Board and Interim CEO with surprisingly no responsibilities related to "oversight of drug pricing, marketing, sales, or distribution," and minimal knowledge of the company.⁵¹²

Mr. Tilles first met Mr. Shkreli when Mr. Shkreli was running a hedge fund.⁵¹³ Around October 2011, Mr. Tilles began to "consult" for Mr. Shkreli's fund MSMB.⁵¹⁴ In December 2011 or January 2012, Mr. Tilles went to Retrophin.⁵¹⁵ At Retrophin, Mr. Tilles was again a consultant working with potential investors.

Mr. Tilles' consulting role at Retrophin appeared to consist of raising capital for Retrophin and serving as a "matchmaker" in potential business deals. He was paid a substantial salary in this role. To this day, Mr. Tilles' home address is in Jensen Beach, Florida, despite the fact that Turing's headquarters is in New York, New York.⁵¹⁶ Although Mr. Tilles would appear on emails with Mr. Urrutia and others on business deals, he had virtually no recollection of those

⁵⁰⁸ *Id.*

⁵⁰⁹ *See, supra*, at 42–45.

⁵¹⁰ *See* Email from Edwin Urrutia to Dan Wichman, TUR-SCA00007881, at TUR-SCA00007881 (May 20, 2015); Email from Edwin Urrutia to Dan Wichman, TUR-SCA00105564, at TUR-SCA00105564 (Jun. 11, 2015); Email from Edwin Urrutia to Dan Wichman, TUR-SCA00105584, at TUR-SCA00105584 (Jun. 11, 2015); Committee Staff Interview with Dan Wichman (Mar. 4, 2016). In the course of the Committee's investigation, and following the intense media scrutiny over the Daraprim price increase, Mr. Wichman also represented to Committee Staff that he declined to invest over concerns that the massive increase Mr. Shkreli contemplated taking on Daraprim would result in harm to patients. Committee Staff Interview with Dan Wichman (Mar. 4, 2016).

⁵¹¹ *See, supra*, at 33–39.

⁵¹² March 2016 Hearing, at 1–2 (written testimony of Ron Tilles).

⁵¹³ *See* Tilles Deposition, at 24:13–20.

⁵¹⁴ *Id.* at 26:5–27:11.

⁵¹⁵ *Id.* at 28:6–13.

⁵¹⁶ *See* Tilles Deposition, at 12:4–8.

deals—his role seemed to be to make introductions to high net worth individuals and push them to invest in Mr. Shkreli’s companies based on material provided by the companies’ business development team. During his deposition, Mr. Tilles admitted that he had no part in any analysis of any deals at either Retrophin or Turing and was merely there to help schedule meetings with investors and function as the conduit between Mr. Shkreli and the investors.⁵¹⁷

Mr. Tilles’ consulting contract with Retrophin was terminated when Mr. Shkreli was ousted at Retrophin. The current CEO of Retrophin, Mr. Aselage, told Committee staff that he viewed Mr. Tilles as an overpaid individual adding no value to the company who viewed it as his right to take extensive trips and bill them to his expense account. Mr. Aselage also stated that Mr. Tilles did not appear to have any knowledge of the pharmaceutical industry, and he thought Mr. Tilles lacked the ability to run a pharmaceutical company.⁵¹⁸

Mr. Tilles followed Mr. Shkreli to Turing and joined its Board. Mr. Tilles recalled at his deposition that Mr. Shkreli simply placed him on Turing’s Board and couldn’t say why he was selected.⁵¹⁹ Subsequent to joining the Board, Mr. Tilles became its Chairman; again, he stated at his deposition that he did not know at first why he was selected for the role.⁵²⁰ While at Turing, Mr. Tilles continued to play the role of investor liaison. Although he appears on most of the emails with Impax during the negotiation of the Daraprim deal, he testified that he merely served as the face of the transaction with Impax and passed information provided by other employees from one company to the other. He never contributed any substantive information about the deal.⁵²¹

Mr. Tilles recalls attending Board meetings but is barely able to relate the substance of Board discussions.⁵²² He stated at his deposition that his role at Board meetings is chiefly that of a figurehead—to call the meetings to order and to vote, although he did not actually come up with ideas for the meetings.⁵²³ When Mr. Shkreli resigned on December 18, 2015, Mr. Tilles was made interim-CEO. It appears to be widely acknowledged, including by Mr. Tilles himself, that he has no pharmaceutical experience other than his time at Retrophin and Turing. At his deposition, he stated that he did not have “a pharma[ceutical] background.”⁵²⁴ Turing’s Board did not ratify the decision to make Mr. Tilles interim-CEO until January 20, 2016 (when Mr. Tilles voted to confirm his own appointment). As CEO, Mr. Tilles appears aloof from the actual running of the company and unaware of key issues faced by pharmaceutical manufacturers.⁵²⁵

The unusual circumstances surrounding Mr. Tilles’ tenure raise the question as to why he was selected to run the company and whether, as Turing’s largest shareholder, Mr. Shkreli installed Mr. Tilles as the interim CEO and Chairman of the Board so that Mr. Shkreli could

⁵¹⁷ *Id.* at 65:13–68:19, 329:4–331:12.

⁵¹⁸ Committee Staff Interview with Steven Aselage (Mar. 10, 2016).

⁵¹⁹ *See* Tilles Deposition, at 65:13–66:15

⁵²⁰ *Id.* at 175:11–177:15

⁵²¹ *Id.* at 83:8–85:14, 103:22–114:3, 190:5–202:18.

⁵²² *Id.* at 134:22–140:23.

⁵²³ Tilles Deposition, at 169:14–173:12.

⁵²⁴ Tilles Deposition, at 334:12–14.

⁵²⁵ *See* Tilles Deposition, at 47:2–48:11.

continue running the company as an activist investor after his resignation as CEO. Turing and Mr. Shkreli have denied this.⁵²⁶

III. Valeant Pharmaceuticals International, Inc.

Valeant Pharmaceuticals International, Inc. is at the center of some of the most visible relationships between a pharmaceutical company and hedge funds. For many of its investors, the company was a Wall Street dream come true. Like the chief executive, Mr. Pearson, many of the top executives hailed from McKinsey, while Mr. Schiller, spent 25 years as an investment banker at Goldman Sachs. Two of the best known names in activist investing—Jeffrey Ubben’s ValueAct and Bill Ackman’s Pershing Square—were some of its largest shareholders.⁵²⁷

Valeant changed from being a typical pharmaceutical company to the form it took with Mr. Pearson at the helm under the guidance of ValueAct, which began investing in Valeant in 2006. ValueAct has held at least one seat on the company’s board since 2007, and more recently held two seats. According to press accounts, ValueAct invested in Valeant because they saw an opportunity to turn the company into a profitable investment through aggressive acquisitions, cutting R&D, and increasing drug prices. These press accounts suggest that ValueAct played a large role in selecting Mr. Pearson as the CEO to oversee these goals and also in designing his compensation package that was based primarily on stock options rather than a base salary as an incentive for Mr. Pearson to push for aggressive returns on investment.⁵²⁸ This may also have incentivized Mr. Pearson to use aggressive accounting practices to reduce Valeant’s effective tax rate to 3.1 percent.⁵²⁹ “As Valeant grew larger, its ties to Wall Street became stronger. From 2013, it generated \$400 million in fees for investment bankers, whose analyst colleagues pumped out research advising investors to snap up the shares.”⁵³⁰

When Valeant began its most aggressive period of acquiring other large companies and drugs, Valeant’s closest investor partner in carrying out that strategy was Mr. Ackman, the Chief Executive Officer and Portfolio Manager of Pershing Square. Without Mr. Ackman, Mr. Pearson would not have had the shareholder support to mount the attempted hostile takeover of Allergan that he did, and its ultimate failure tied Valeant and Pershing Square together for the long-haul.⁵³¹

A. Pershing Square

⁵²⁶ See Katie Thomas and Andrew Pollack, *Turing Pharmaceuticals Accused of Retaliating for Sex Assault Complaint*, N.Y. Times (Aug. 23, 2016); Tilles Deposition, at 271:7-17 and 283:4-12.

⁵²⁷ See David Crow, *Valeant—The Harder They Fall*, Financial Times (Mar. 28, 2016).

⁵²⁸ See Andrew Ross Sorkin, *ValueAct Pays a Price for Its Supporting Role at Valeant*, N.Y. Times (Mar. 28, 2016); Maureen Farrell, *ValueAct’s Not-So-Invisible Role in Valeant’s Business and Pay Model*, Wall St. J. (Mar. 21, 2016).

⁵²⁹ See Andrew Ross Sorkin, *Do Drug Companies Make Drugs, or Money?*, N.Y. Times (June 2, 2014).

⁵³⁰ David Crow, *Valeant—the Harder They Fall*, Financial Times (Mar. 28, 2016).

⁵³¹ See David Gelles, *Valeant and Ackman Departing from Usual Playbook in Pursuit of Allergan*, N.Y. Times (June 2, 2014); David Gelles, *No Allergan Deal, but a \$2.6 Billion Profit for Ackman*, N.Y. Times (Nov. 17, 2014).

Pershing Square's relationship with Valeant began in early February 2014, when Mr. Bill Doyle, a member of Pershing Square's Investment team and a former colleague of Mr. Pearson's at McKinsey, introduced Mr. Ackman and Mr. Pearson.⁵³² Shortly thereafter, Mr. Ackman and Mr. Pearson agreed to form a partnership between Pershing Square and Valeant to launch an offer for Allergan, a large pharmaceutical company and manufacturer of Botox.⁵³³ Such an alliance between an activist investor like Pershing Square and a corporate acquirer like Valeant was unusual.⁵³⁴

As a result of the attempted Allergan offer, a process which spanned from February 2014 through November 2014, Pershing Square acquired an in-depth knowledge of Valeant's operations.⁵³⁵ Prior to Pershing Square and Valeant's agreement regarding the Allergan offer, Pershing Square conducted due diligence on Valeant but did not flag Valeant's drug pricing strategy as an area of concern.⁵³⁶ During the course of the Allergan offer, Mr. Ackman and Mr. Pearson frequently spoke daily.⁵³⁷

1. Pershing Square Becomes a Top Valeant Shareholder

Following the failed Allergan offer, Pershing Square became a direct investor in Valeant.⁵³⁸ For much of 2015, Mr. Ackman stated that he considered Pershing Square to be a "passive investor" in Valeant.⁵³⁹ During this period, however, Mr. Ackman and Mr. Pearson still spoke "frequently," according to Pearson.⁵⁴⁰ The frequent communications between the two is also reflected in documents produced to the Committee, in which Mr. Ackman frequently discusses his attempts to prop up Valeant in the media as well as with other investors, such as Berkshire Hathaway.⁵⁴¹

For example, on March 4, 2015, Mr. Ackman personally contacted Mr. Schiller to inform him that Pershing Square had become "a top 5 shareholder" in Valeant.⁵⁴² At this time, Mr. Ackman requested a meeting with Mr. Schiller and Mr. Pearson, in which Mr. Ackman later proposed that Pershing Square and Valeant create "a stake building fund."⁵⁴³ According to Mr. Pearson, the stake building fund was intended to be a second investment vehicle by which the

⁵³² Committee Staff Interview with William B. Ackman (Apr. 7, 2016) ("Ackman Interview").

⁵³³ See Michele Celarier, *Bill Ackman and Michael Pearson: The Inside Story*, Fortune (Mar. 27, 2016).

⁵³⁴ See David Benoit, Dana Mattidi, & Jonathan D. Rackoff, *Ackman, Valeant Team Up to Pursue Takeover of Allergan*, Wall St. J. (April 21, 2014).

⁵³⁵ Ackman Interview.

⁵³⁶ *Id.*

⁵³⁷ See Pearson Deposition, at 308:16–22.

⁵³⁸ *Id.* at 311:7–16.

⁵³⁹ Ackman Interview.

⁵⁴⁰ Pearson Deposition, at 308:23–309:3.

⁵⁴¹ Email from William A. Ackman to J. Michael Pearson and Howard Schiller, VRX_SCA_A_00003377, at VRX_SCA_A_000003377–78 (Apr. 10, 2015); Email from William A. Ackman to J. Michael Pearson and Howard Schiller, VRX_SCA_A_00003381, at VRX_SCA_A_00003381–82 (Apr. 11, 2015); Email from William A. Ackman to Charlie Munger, VRX_SCA_A_00003385 (Apr. 11, 2015).

⁵⁴² Email from Howard Schiller to J. Michael Pearson, VRX_SCA_A_00022687 (Mar. 4, 2015).

⁵⁴³ Email from William A. Ackman to J. Michael Pearson, VRX_SCA_A_00003388 (Apr. 19, 2015); Pearson Deposition, at 313:8–314:14 and 317:14–318:2.

two companies could make additional hostile takeover attempts.⁵⁴⁴ Mr. Pearson ultimately brought Mr. Ackman's idea regarding the stake building fund to Valeant's Board, but the Board chose not to pursue it.⁵⁴⁵ On March 25, 2015, Pershing Square filed a Schedule 13D with the SEC, stating that it had acquired a 5.7 percent interest in Valeant.⁵⁴⁶

Before Pershing Square gained a seat on Valeant's Board, Mr. Ackman testified that Valeant's price increases on individual drugs were not transparent to him as only a shareholder.⁵⁴⁷ Nevertheless, on July 23, 2015, nearly seven months before Pershing Square gained a seat on Valeant's Board, Mr. Ackman emailed Mr. Pearson following a 2015 second quarter call Valeant held that day to tell him that he sounded "a little defensive" on "the price increase question" and could have "answered that a little differently."⁵⁴⁸ In the same email, Mr. Ackman also wrote:

I can't think of a business over the course of my career that has delivered such strong operating performance, BD performance, and participates in such a large market where the competitors in most cases don't compare. That combined with transparency, accountability, and shareholder orientation is unique, particularly at the current scale of VRX.

Mr. Ackman closed the email by saying, "Thank you for delivering on our behalf."⁵⁴⁹ This is one of many such emails between Mr. Ackman and Mr. Pearson.

2. Pershing Square Response to Philidor Allegations

Until January 2016, Valeant maintained an opaque relationship with Philidor, a mail-order pharmacy, for which it is now under investigation. Valeant claims that its relationship with Philidor helped it to retain patients.⁵⁵⁰ Philidor claims that Valeant merely used it to operate Valeant's PAP.⁵⁵¹ On November 17, 2016, Gary Tanner, a former executive at Valeant, and

⁵⁴⁴ Pearson Deposition, at 313:8–314:14 and 317:14–318:2.

⁵⁴⁵ *Id.* at 313:8–318:2.

⁵⁴⁶ See Pershing Square, Schedule 13D (Mar. 25, 2015), *found at*, <http://www.sec.gov/Archives/edgar/data/885590/000119312515104528/d892712dsc13d.htm> (last visited Dec. 16, 2016). Schedule 13D is commonly referred to as a "beneficial ownership report." When a person or group of persons acquires beneficial ownership of more than 5% of a voting class of a company's equity securities, they are required to file a Schedule 13D with the SEC.

⁵⁴⁷ April 2016 Hearing, Trans. at 69:17–71:4.

⁵⁴⁸ Email from William A. Ackman to J. Michael Pearson, VRX_SCA_A_00000029 (July 23, 2015)

⁵⁴⁹ *Id.*

⁵⁵⁰ See Pearson Deposition, at 146:19–147:3.

⁵⁵¹ See Senate Special Committee on Aging, Philidor Interrogatories (April 25, 2016);

Andrew Davenport, the former CEO of Philidor, were indicted for allegedly illegally using Philidor in a kickback scheme to convert Valeant shareholder money into personal profit.⁵⁵²

Allegations were reported in the media that Valeant used Philidor to artificially boost its sales numbers, and that Philidor changed doctor's prescriptions to Valeant's brand-name drugs.⁵⁵³ Valeant denied these allegations but admitted that it purchased an option to buy Philidor for \$100 million two years ago and also included Philidor's accounting in Valeant's books even though it had not exercised the option to purchase Philidor.⁵⁵⁴ Valeant severed its ties with the now-defunct Philidor in October of last year.⁵⁵⁵ The failure to disclose its relationship with Philidor is being investigated by the U.S. Attorney for the Southern District of New York.⁵⁵⁶ Valeant is also being investigated by the U.S. Securities and Exchange Commission, and the U.S. Attorney for the District of Massachusetts, among others.⁵⁵⁷ In answers to written deposition questions provided to the Committee, Mr. Schiller said that Mr. Pearson did not reveal the relationship in part because he believed it gave Valeant an advantage over its competitors.⁵⁵⁸

After much media scrutiny, Valeant cut ties with Philidor. The Valeant Board of Directors formed a special committee to investigate whether there were any improprieties with the relationship.⁵⁵⁹ At the end of the investigation, the Committee determined that Valeant will restate \$58 million in financial earnings from late 2014 into 2015.

Pershing Square's response to the public scrutiny of Philidor illustrates Mr. Ackman's active involvement in Valeant. Following the revelations about Valeant's relationship with Philidor, Mr. Ackman and Pershing Square attempted "to salvage his huge bet on Valeant" by playing their "traditional" activist investor role with respect to the company.⁵⁶⁰ In doing so, Pershing Square sent Valeant their opinion on what should be included in Valeant investor calls as well as questions that Valeant should be prepared to answer, and asked to review press

⁵⁵² See Department of Justice, *Former Valeant Executive and Former Philidor CEO Charged in Manhattan Federal Court for Illegal Fraud and Kickback Scheme*, at _ (Nov. 17, 2016), found at, <https://www.justice.gov/usao-sdny/pr/former-valeant-executive-and-former-philidor-ceo-charged-manhattan-federal-court> (last visited Dec. 16, 2016).

⁵⁵³ See Caroline Chen and Ben Elgin, *Philidor Said to Modify Prescriptions to Boost Valeant Sales*, Bloomberg (Oct. 29, 2015, updated Oct. 30, 2015), found at, <https://www.bloomberg.com/news/articles/2015-10-29/philidor-said-to-modify-prescriptions-to-boost-valeant-sales> (last visited Dec. 16, 2016).

⁵⁵⁴ *Id.*

⁵⁵⁵ See Caroline Chen, *Valeant Falls on Report of Criminal Probe Into Philidor Ties*, Bloomberg (Aug. 10, 2016), found at, <https://www.bloomberg.com/news/articles/2016-08-10/valeant-said-to-be-under-criminal-investigation-wsj-reports-irpjbydi> (last visited Dec. 16, 2016).

⁵⁵⁶ *Id.*

⁵⁵⁷ *Id.*

⁵⁵⁸ See Deposition of Mr. Howard Schiller, Answer to Written Question Number 9 (Apr. 11, 2016). Mr. Schiller also noted that this decision was reported to the Valeant Audit Committee.

⁵⁵⁹ See Valeant, *Valeant Ad Hoc Committee Has Made Substantial Progress in Its Review of Philidor and Related Accounting Matters*, Valeant (February 22, 2016).

⁵⁶⁰ Monica Langley, *Activist Investor Bill Ackman Plays Defense*, The Wall Street Journal (Nov. 4, 2015) found at <http://www.wsj.com/articles/activist-investor-bill-ackman-plays-defense-1446689963> (last visited Dec. 16, 2016).

releases before Valeant put them out.⁵⁶¹ Pershing Square also undertook their own survey of dermatologists to collect their views on Valeant products and the company's use of Philidor.⁵⁶²

Further, Mr. Ackman began speaking regularly with the press about the Philidor scandal and making public his recommendations to Valeant. The night before the *Wall Street Journal* published a page-one story, Mr. Ackman emailed Mr. Pearson to say that he had “done [his] best to make it a good story and for you and the company to look as good as possible. Fingers crossed.”⁵⁶³ Despite Mr. Ackman's efforts, the story did not portray Valeant positively and Mr. Ackman emailed Mr. Pearson and others at Valeant the next night, stating, “It is always a debate as to whether to work with the press or not, but I chose to do so with the Journal. I did my best to get the article to a good place. Clearly, this was not my best work. My apologies.”⁵⁶⁴ According to Mr. Pearson and Mr. Schiller, Mr. Ackman's public relations strategy conflicted with Valeant's, straining the relationship between Pershing Square and Valeant.⁵⁶⁵

Even as Mr. Ackman escalated his public criticism of Valeant, he continued to be privately and publicly supportive of Mr. Pearson as CEO. In a private email to Mr. Pearson on October 29, 2015, Mr. Ackman stated, “I just want you to know that I am totally supportive of you as CEO of Valeant I just want to make sure you don't confuse my disagreement with you about a conference call with my confidence in you as CEO.”⁵⁶⁶ In an email sent to Mr. Pearson on November 5, 2015, Mr. Ackman stated:

While I have strong views on Valeant's communication strategy and would have taken a different approach, you and the board should not interpret this as a negative reflection on my view of you as the CEO of the company You are one of the most shareholder-oriented CEOs I know. You have assured me that you and the rest of the board are considering any and all alternatives that would benefit shareholders and other stakeholders. That is very comforting to us.⁵⁶⁷

Publicly, on November 9, 2015, during a Pershing Square quarterly investor call, Mr. Ackman stated, “[t]he biggest regret I have with Valeant is that we're not in a position to buy more.”⁵⁶⁸ On November 23, 2015, Pershing disclosed that it had nearly doubled its interest in Valeant—increasing its ownership of the company from 5.7 percent of the company to 9.9 percent.⁵⁶⁹

⁵⁶¹ See Email from Jordan Rubin to Mike Pearson, VRX_SCA_A_00005820 (Oct. 17, 2015), Email from William A. Ackman to Howard Schiller and Mike Pearson, VRX_SCA_A_00005936 (Oct. 22, 2015), Email from William A. Ackman to Mike Pearson, Laurie Little, and Robert Chai-Onn, VRX_SCA_A_00005829 (Oct. 25, 2015), Email from William A. Ackman to Mike Pearson, and Howard Schiller, VRX_SCA_A_00005848 (Oct. 22, 2015).

⁵⁶² See Email from Jordan Rubin to Mike Pearson and Andrew Davis (Nov. 9, 2015), VRX_SCA_A_00005999-6000.

⁵⁶³ Email from William A. Ackman to Mike Pearson (Nov. 4, 2015), VRX_SCA_A_00005994.

⁵⁶⁴ Email from Mike Pearson to William Ackman (Nov. 6, 2015), VRX_SCA_A_00005997.

⁵⁶⁵ See Schiller Deposition, at 125:9-130:7; Pearson Deposition, at 272:18-273:17.

⁵⁶⁶ Email from William A. Ackman to Mike Pearson (Oct. 29, 2015), VRX_SCA_A_00005983.

⁵⁶⁷ Email from William A. Ackman to Mike Pearson (Nov. 5, 2015), VRX_SCA_A_00005995.

⁵⁶⁸ Mittelman, Melissa. *Ackman's Biggest Valeant Regret Is Being Unable to Buy More*, Bloomberg (November 9, 2015).

⁵⁶⁹ See Pollock, Lauren. *Ackman's Pershing Square Boosts Stake in Valeant to 9.9%*, Wall St. J. (Nov. 23, 2015).

Then on December 31, 2015, Mr. Ackman sold about five million shares in Valeant in order to generate a tax loss as investors in Pershing faced the biggest loss in the company's history. At the end of 2015, Pershing Square shares in Valeant were down to 8.5 percent of the company.⁵⁷⁰

3. Pershing Square Gains Two Valeant Board Seats

On February 29, 2016, the day after Valeant announced Mr. Pearson would return from medical leave, Mr. Ackman visited Mr. Pearson at Valeant to request that Stephen Fraidin, Vice Chairman of Pershing Square, be made a member of Valeant's Board of Directors.⁵⁷¹ Mr. Pearson instructed Mr. Ackman to bring his request to Robert Ingram (then Chairman of the Board) for the Board to consider.⁵⁷² In an email sent by Mr. Ackman to Mr. Pearson following his February 29 visit to Valeant, Mr. Ackman stated, "[w]e greatly appreciate the work you are doing on behalf of us and the other shareholders . . . You come across very well and I think will be very comforting for analysts."⁵⁷³

On March 8, 2016, the Board voted to make Mr. Fraidin a Valeant Director.⁵⁷⁴ The same day, Mr. Ackman emailed Mr. Pearson, stating, "[t]here isn't enough time for your team to review our agreement requests prior to Steve joining the board so we are going to drop the requests and we can deal with them later."⁵⁷⁵ According to Mr. Pearson, Mr. Ackman's requests were "largely legalese" and "were around trading," primarily "when someone could trade and not trade."⁵⁷⁶

On March 16, 2016, Mr. Ackman contacted Mr. Pearson and asked him if he could speak to Valeant's Board for three to four hours during the upcoming Valeant Board meeting, which commenced the following day.⁵⁷⁷ Mr. Pearson relayed this request to the Board and the next day, Mr. Ackman joined the Board meeting as an observer through the weekend.⁵⁷⁸ On March 18, 2016, Mr. Ackman requested that the Board make him a Director.⁵⁷⁹ According to Mr. Pearson, when Mr. Ackman made the request, he stated that he "had 100 percent confidence" in Mr. Pearson as CEO.⁵⁸⁰ However, on March 20, 2016, Mr. Ackman called Mr. Pearson to inform him that the Board will vote on replacing him as CEO.⁵⁸¹ On March 21, 2016, it was

⁵⁷⁰ See Herbst-Bayliss, Svea, *Ackman's Fund Sells 5 Million Valeant Shares to Generate Tax Loss*, Reuters (Dec. 31, 2015).

⁵⁷¹ See Pearson Deposition, at 332:18–333:22.

⁵⁷² See Pearson Deposition, at 333:17–334:8.

⁵⁷³ Email from William A. Ackman to Mike Pearson, VRX_SCA_A_00012758. (Feb. 29, 2016).

⁵⁷⁴ Valeant, *Valeant Pharmaceuticals Announces The Addition Of Three New Independent Directors To Its Board* (Mar. 9, 2016), found at, <http://ir.valeant.com/news-releases/2016/03-09-2016-130553844> (last visited Dec. 16, 2016).

⁵⁷⁵ Email from William A. Ackman to Mike Pearson, VRX_SCA_A_00022207 (Mar. 8, 2016).

⁵⁷⁶ Pearson Deposition, at 336:9–17.

⁵⁷⁷ See Pearson Deposition, at 338:12–17.

⁵⁷⁸ *Id.* at 338:12–339:13; see also, Committee Staff Interview with Bill Ackman (Apr. 7, 2016)

⁵⁷⁹ *Id.*

⁵⁸⁰ Pearson Deposition, at 344:8–17.

⁵⁸¹ See Pearson Deposition at 339:23–345:4.

reported that Valeant's Board voted to add Mr. Ackman as a Director and to dismiss Mr. Pearson as CEO.⁵⁸²

When asked when Mr. Ackman lost confidence in him as CEO, Mr. Pearson replied during his deposition, "[s]ometime between Friday and Sunday of that weekend."⁵⁸³ As to why Mr. Ackman asked Mr. Pearson to step down as CEO, Mr. Pearson opined, "I think it had to do primarily with the share-price performance and that he was probably feeling a lot of heat from his investors and, therefore, if you're an activist, you make changes. And a change to the CEO is probably the easiest change to make."⁵⁸⁴ During the Committee's call with Mr. Ackman, he would not comment on the Board discussions around the decision to ask Mr. Pearson to step down as CEO. He acknowledged, however, that he is very involved in the company's direction moving forward.⁵⁸⁵

IV. Rodelis Therapeutics

Of the four companies that were the subject of the Committee's investigation, Rodelis Therapeutics exhibited the least separation between the company and its investors. During its existence as Rodelis, the company maintained no discernable separation from its largest investor, Avego Healthcare Capital. In fact, the two companies even shared a mailing address at one point.⁵⁸⁶

The core Rodelis team included Mr. Venkataraman, the founder of Avego Healthcare Capital, and Mr. Goldstein, a partner at Avego.⁵⁸⁷ It is apparent from internal Rodelis documents that virtually no effort was made either to separate Rodelis from Avego, or to separate the roles of individuals holding senior office in both companies.

Both Mr. Venkataraman and Mr. Goldstein were involved in detailed analyses regarding Rodelis' acquisition of cycloserine/Seromycin.⁵⁸⁸ Although Scott Spencer was the General Manager of Rodelis, he was brought on only in late 2015 and his role appeared to be relegated to dealing with the outward facing backlash that occurred after the price increase, and he was absent from key decision-making emails.⁵⁸⁹ Mr. Goldstein was an active driver in the decisions involving Rodelis and Seromycin throughout 2015, maintaining and using both an Avego and a Rodelis email throughout the transaction for Seromycin.⁵⁹⁰

⁵⁸² Committee Staff Interview with Bill Ackman (Apr. 7, 2016).

⁵⁸³ Pearson Deposition, at 344:8-17.

⁵⁸⁴ Pearson Deposition, at 344:18-345:4.

⁵⁸⁵ Steele, et al, *Valeant Names Joseph Papa as New CEO*, Wall Street Journal (Apr. 25, 2016).

⁵⁸⁶ See Email from Srihari Vedartham to Dr. Stowell, RTI-USSSCA-00002461 (Apr. 3, 2015); Email from Michael Goldstein to Jacinta McCabe, Ciaran Lyng, Sarah Cleary, Jim Clery, and Brian Kelly, RTI-USSSCA-00000277 at RTI-USSSCA-00000279-81 (Aug. 7, 2015).

⁵⁸⁷ See Rodelis Therapeutics Overview, RTI-USSSCA-00000321, at RTI-USSSCA-00000325

⁵⁸⁸ See Email from Virinder Nohria to Bala Venkataraman and John Devane, RTI-USSSCA-00000423 (June 15, 2015).

⁵⁸⁹ See Email from Srihari Vedartham to Michael Goldstein, RTI-USSSCA-00007233 (July 14, 2015); Email from Scott Spencer to Andrew Pollack, RTI-USSSCA-00005071 (Sept. 15, 2015).

⁵⁹⁰ See Email from Brian Jenette to Michael Goldstein, RTI-USSSCA-00007974 (July 27, 2015); Email from John Devane to Michael Goldstein, RTI-USSSCA-00000276 (July 28, 2015).

In several instances, Mr. Goldstein, using his Avego email address, was directly involved in directing regulatory, payments, and pricing analysis on Seromycin prior to the purchase.⁵⁹¹

Even after Rodelis closed on the Seromycin deal, Mr. Goldstein continued to direct ongoing pricing analysis into the product, giving no indication that his involvement in the Seromycin project while being a partner at Avego was going to wane in the future.⁵⁹² Avego also ordered, under its own name and not that of Rodelis, pricing and payer research on a number of drugs, including Seromycin.⁵⁹³

As seen in the above discussion of the other three companies, activist investors maintained close relationships with the executives of the companies they invested in, particularly those which have the largest share of a company as Avego did with Rodelis. However, even the most active investors in the other examples maintained a veneer of detachment in that, no matter how hard they may have pushed and pressured the executives of the companies they invested in to take the companies in a direction that was best for the activist investors, ultimately, the executives of the companies had final say in management decisions. In the case of Avego, investors made no attempt to hide the fact that they were running Rodelis. This is a prime example of the control exercised by some investors in healthcare at the cost of patients.

⁵⁹¹ See Email from Michael Goldstein to Kevin Rohrbach (Aug. 26, 2015), RTI-USSSCA-00008039; Email from Srihari Vedartham to Michael Goldstein, RTI-USSSCA-00007233 (July 14, 2015); Email from Kevin Rohrbach to Michael Goldstein, RTI-USSSCA-00007980 (August 20, 2015); Email from Colin Shannon to Michael Goldstein and Srihari Vedartham, RTI-USSSCA-00007871 (July 22, 2015); Email from Michael Goldstein to Bala Venkataraman and Srihari Vedartham, AVEGO-00000883 (July 30, 2015); Email from Brian Jennette to Michael Goldstein, AVEGO-00000965 (August 6, 2015).

⁵⁹² See Email from Michael Goldstein to Kevin Rohrbach (Aug. 26, 2015), RTI-USSSCA-00008039.

⁵⁹³ See Avego Healthcare [redacted] and Cycloserine Research (August 2015), RTI-USSSCA-00001248-58; Cycloserine Research (August 2015), RTI-USSSCA-00001259-1264.

CHAPTER 5. HOSPITAL, PATIENT, AND COMMUNITY IMPACTS

I. Harm to Patients and Their Families

Based on its extensive study, the Committee believes that sudden drug price increases have imposed substantial burdens on patients and their families. Dramatic price hikes are affecting their health, time, emotional well-being, and pocketbooks. According to the many patient accounts obtained by the Committee, these impacts are often interlinked.⁵⁹⁴ In some cases, patients are forced to go without vital medicine, and experience dangerous and sometimes life-threatening symptoms as a result. In others, patients—and often their families and physicians—reported having to skip doses or hoard pills out of fear that their next refill would not be available or would be unaffordable.⁵⁹⁵ Physicians bemoaned the effects that poor adherence to prescribed dosing has had on their patients’ health.⁵⁹⁶ Even patients who obtained medication through PAPs or who still had insurance coverage for their medication reported watching anxiously as prices climbed, knowing that they could lose access without warning if the drug were dropped from their insurance plan’s formulary at any time throughout the year, or if their application for patient assistance were denied at any point.⁵⁹⁷

The Committee’s interviews reveal that families, seeing their loved ones declining and scared, often feel powerless in the face of drug price increases, but also become advocates, making calls or scouring the Internet in search of alternative or lower cost therapies.⁵⁹⁸ Family budgets are stressed even when patients get help from a PAP or have what they consider to be good insurance coverage.⁵⁹⁹ Some family members reported taking a second job on top of taking on increased caretaking responsibilities at home.⁶⁰⁰ Others navigated through vague information on websites and applied to multiple PAPs or grants in search of one that would cover their needs. Several individuals likened the paperwork requirements—that require continually reapplying and following up—to having a part-time job.⁶⁰¹

⁵⁹⁴ Committee Staff Interview with Patient A (PII [UNDER SEAL]) (Mar. 24, 2016) (“Patient A Interview”); Committee Staff Interview with Susan Mannes (wife of patient Bruce Mannes) (Mar. 24, 2016) (“Mannes Interview”); Committee Staff Interview with Patient B (PII [UNDER SEAL]) (Apr. 4, 2016) (“Patient B Interview”). Committee Staff interviewed a number of patients and family members affected by the price increases in the seven drugs subject to the Committee’s investigation. Some of these individuals (understandably) requested anonymity regarding *public* attribution of their statements. The names of these individuals have been anonymized. The Committee is not relying on anonymous reports, as all anonymized names are known to the Committee, and Committee Staff interviews with those individuals were subject to 18 U.S.C. §§ 1001 and 1505. Full notes of these interviews are contained in the Committee’s sealed files.

⁵⁹⁵ Committee Staff Interview with Dr. Michael Schilsky (Mar. 23, 2016) (“Schilsky Interview”).

⁵⁹⁶ *Id.*

⁵⁹⁷ Patient A Interview, Patient B Interview; Mannes Interview; Committee Staff Interview with Trisha Marzolo (mother of patient Patrick Melvin) (Mar. 28, 2016) (“Marzolo Interview”).

⁵⁹⁸ *Id.*

⁵⁹⁹ Mannes Interview.

⁶⁰⁰ *Id.*

⁶⁰¹ *Id.*

II. Drug-Specific Patient Impacts

A. Wilson Disease Drugs: Cuprimine and Syprine

Valeant's sudden price increases for Cuprimine and Syprine have hit patients particularly hard due to the nature of treating Wilson disease, which results in the inability to process copper and requires treatment for the rest of a person's life.⁶⁰² For decades, individuals with Wilson disease have relied on Cuprimine and Syprine to lead otherwise ordinary lives. Failure to treat Wilson disease is not an option. Dr. Askari, director of the Wilson Disease Center of Excellence at the University of Michigan Health System, who has overseen the care of hundreds of patients, testified before the Committee on April 27 that:

Wilson disease is completely manageable with proper treatment; however, it is a uniformly fatal disease if left untreated. It can be a crippling disease if copper levels are not well controlled or if the diagnosis is not made early enough Risks of not treating Wilson disease or gaps in treatment include liver failure, brain damage, and death.⁶⁰³

The price increases for Cuprimine and Syprine resulted in interruptions in treatment, difficulty accessing medication, and the return of painful symptoms for patients. Writing in the journal *Hepatology* in April 2015, prominent Wilson disease specialists reported that price increases were beginning to create financial crises for many patients and that further serious personal health crises were looming.⁶⁰⁴

Dozens of Americans affected by Wilson disease contacted the Committee over the course of the investigation to share their stories and struggles. Patients ranged from newly diagnosed young adults scrambling to make ends meet, to seniors facing retirement. The older adults had typically been successfully managing their disease with Cuprimine or Syprine for most of their lives, and the sudden price hikes presented an acute risk to their life, health, and wellbeing. Even those who received foundation grants for co-pays or enrolled in a PAP still faced burdensome out-of-pocket costs.⁶⁰⁵ While some patients were able to eventually get assistance in obtaining the medication they needed at an affordable price, many went without medication for a period of time, thus jeopardizing the management of their condition.⁶⁰⁶ Other patients decided to switch to zinc acetate, a treatment that poses risks for some patients.⁶⁰⁷

All of the Wilson disease sufferers that the Committee interviewed, including those eventually able to obtain financial assistance for the drugs, were deeply anxious about long-term

⁶⁰² Wilson disease is also referred to by some in the medical community as Wilson's disease.

⁶⁰³ April 2016 Hearing, at 1 (written testimony of Dr. Frederick Askari, M.D.).

⁶⁰⁴ See Michael J. Schilsky et al., *Costly Choices for Treating Wilson's Disease*, *Hepatology* Vol. 61, No. 4, at 1107 (2015).

⁶⁰⁵ Patient A Interview; Patient B Interview; Email with Patient B (PII [UNDER SEAL]) (Apr. 11, 2016).

⁶⁰⁶ Marzolo Interview; Patient B Interview; Email with Patient B (PII [UNDER SEAL]) (Apr. 11, 2016).

⁶⁰⁷ See, *supra*, at 8.

affordability and access.⁶⁰⁸ Physicians emphasized that emotional effects can exacerbate a patient's physical symptoms.⁶⁰⁹ Some even expressed concern that speaking publicly would lead Valeant to stop manufacturing the drugs altogether, while others were angry over what they viewed as Valeant's ability to "hold them hostage."⁶¹⁰ Some patients were disturbed that Valeant was profiting from the price increases while claiming publicly to be helping people through its PAP.⁶¹¹ Some patients were only offered assistance from Valeant after speaking about their challenges to the press.⁶¹²

Below are the stories of three Wilson disease patients who testified before or were interviewed by the Committee.

- In the Committee's April 27, 2016, hearing, Berna Heyman, retired Associate Dean of Libraries at the College of William and Mary, shared her experience struggling to treat her Wilson disease. Prior to the price hikes, Mrs. Heyman took Syprine three times a day. In 2014, she determined that her projected co-pay would exceed \$10,000 per year—with her insurance paying over \$260,000—and realized that such costs were untenable for her (despite having an objectively "good" insurance plan).⁶¹³ Mrs. Heyman explained the trials that followed: she applied for Valeant's PAP, and was denied assistance; she wrote to the then-Valeant CEO, Michael Pearson, who responded that the price increases were necessary to support Valeant's overall activities; she applied to the Patient Access Network Foundation, and was told that her income precluded her from obtaining support.⁶¹⁴ Ultimately, Mrs. Heyman switched to a zinc-based drug, but in doing so, she endures lifestyle restrictions and uncertainties about future effectiveness.⁶¹⁵ She testified, "[m]y health was stable with Syprine and my doctor and I made the change only under duress."⁶¹⁶

Mrs. Heyman also testified and told Committee staff that a year after she had stopped taking Syprine, reporters from major newspapers contacted her, and then talked to Valeant about her case.⁶¹⁷ Following these interviews, Mrs. Heyman was contacted by a Valeant representative offering to enroll her in a PAP as an exception to the program's requirements.⁶¹⁸ Mrs. Heyman refused Valeant's offer, maintaining that the drug should be offered to all patients at an affordable cost.⁶¹⁹ Mrs. Heyman later testified that after this offer, Valeant sent flowers with a note saying it was a

⁶⁰⁸ Patient A Interview; Manness Interview; Marzolo Interview; Heyman Interview.

⁶⁰⁹ Committee Staff Interview with Dr. Frederick Askari (Mar. 28, 2016).

⁶¹⁰ Patient A Interview; Patient B Interview.

⁶¹¹ Patient A Interview.

⁶¹² *See, infra*, at 100.

⁶¹³ April 2016 Hearing, at 1–2 (written testimony of Berna Heyman).

⁶¹⁴ *Id.* at 2.

⁶¹⁵ *Id.*

⁶¹⁶ *Id.*

⁶¹⁷ *Id.*

⁶¹⁸ *Id.*

⁶¹⁹ *Id.* at 3.

pleasure to talk to her. Mrs. Heyman testified, “I refused the flowers and asked that the sender be informed of my refusal.”⁶²⁰

- A retired carpenter from Michigan, Bruce Mannes, shared his story in the *New York Times* and later his wife spoke with the Committee.⁶²¹ Mr. Mannes had been managing his Wilson disease well for 55 years with Cuprimine, until the summer of 2015, when his monthly co-pay rocketed from about \$366 to \$1,800.⁶²² The price hike caused the Mannes family substantial stress, and Mr. Mannes’s wife took on a second part-time job to help cover the added expenses, stating unequivocally, “my husband will die without the medicine.”⁶²³ Following the *New York Times* story, Valeant contacted the couple and made an exception to its PAP requirements, providing the drug directly to Mr. Mannes at no cost.⁶²⁴
- The Committee also heard the story of Patrick Melvin, a young father. Mr. Melvin was diagnosed with Wilson disease in July 2014 and was able to continue leading a normal life with the disease controlled by Syprine. When his insurance company reduced the amount it would cover, leaving him a co-pay of \$20,000 for a month’s supply, he went without the drug for several weeks and his symptoms escalated to where he began to have increased tremors and hallucinations, and to slur words, drool, and lose his memory. His mother’s efforts to find assistance were eventually successful, but Mr. Melvin’s health had declined and was forced to apply for disability assistance. With changing employment and income status due to his declining health, his eligibility for assistance programs also changed, which required his mother to continue navigating these programs on his behalf. Although Mr. Melvin improved with continuing medical treatment and was rebuilding his skills, including caring for his young daughter, he died tragically after suffering a massive stroke in September 2015. He was 35 years old.⁶²⁵

In meeting with Committee staff on August 30, 2016, current Valeant CEO Joseph Papa told Committee staff that Valeant had not reduced the prices of Cuprimine and Syprine or provided rebates, and did not have plans to do so. According to Mr. Papa, the company was instead focusing on ensuring that patients can obtain these drugs by making changes to its PAPs. Specifically, Mr. Papa said that Valeant had expanded the coverage of its PAP to ensure that no patient with insurance has a co-pay of more than \$25, and that a patient without insurance with a household income of less than 500 percent of the federal poverty level would receive free medication.⁶²⁶

⁶²⁰ *Id.*

⁶²¹ Andrew Pollack & Sabrina Tavernise, *Valeant’s Drug Price Strategy Enriches it, But Infuriates Patients and Lawmakers*, N.Y. Times (Oct. 4, 2015).

⁶²² *Id.*

⁶²³ *Id.*

⁶²⁴ Mannes Interview.

⁶²⁵ Marzolo Interview; Committee Staff Interview with Dr. Laurice Yang (doctor treating Patrick Melvin) (Apr. 21, 2016); Committee Staff Interview with Joey Gee (doctor treating Patrick Melvin) (Apr. 1, 2016); Committee Staff Interview with Dr. Jeff Bronstein (doctor treating Patrick Melvin) (Mar. 31, 2016) (“Bronstein Interview”).

⁶²⁶ Papa Interview; Valeant, Responses to Senate Special Committee on Aging, at 2 (Sept. 13, 2016).

B. Toxoplasmosis Drug: Daraprim

As discussed previously, toxoplasmosis is a parasitic infection mainly affecting patients with compromised immune systems.⁶²⁷ Infants are at risk because their immune systems are not sufficiently developed to combat the infection.⁶²⁸ Most people with healthy immune systems who contract toxoplasmosis feel mildly ill, if they feel ill at all.⁶²⁹ For those at risk, however, toxoplasmosis can cause brain and organ damage and result in blindness or death if not properly treated.⁶³⁰

Many Americans with toxoplasmosis are among the most vulnerable of patients, with limited financial resources and limited support systems.⁶³¹ Patients in the advanced stages of AIDS are among those at high risk to develop toxoplasmosis.⁶³² Patients who seek treatment typically present with a brain abscess causing seizures and rapidly declining function, which can subsequently quickly deteriorate to death.⁶³³ They are stabilized in the hospital and discharged with a prescription to continue taking Daraprim at home.⁶³⁴

For toxoplasmosis patients, the turmoil created by Turing's purchase of Daraprim in August 2015 and its immediate price hike from \$1,350 to \$75,000 for a bottle of 100 pills was especially threatening to their health. Dr. Adaora Adimora, a physician and professor at the University of North Carolina, testified before the Committee in March 2016 that as a result of Turing's price hikes, patients had experienced treatment interruptions or delays, and some had gone without treatment entirely.⁶³⁵ Following the price hikes, some insurance companies made it much more difficult for their beneficiaries to access Daraprim.⁶³⁶ In interviews with the Committee, health care providers noted that PAPs and non-profit grants often fail to provide benefits for these patients, are difficult to navigate, and are not well advertised.⁶³⁷

Dr. Adimora reported further that patients and providers were battling insurance companies, searching for financial help, and in some cases turning to drug compounding or alternative therapies.⁶³⁸ She and a number of other physicians, hospital administrators, and

⁶²⁷ See, *supra*, at 33.

⁶²⁸ See March 2016 Hearing, at 2 (written testimony of Adaora Adimora, M.D.).

⁶²⁹ See Centers for Disease Control, *Parasites—Toxoplasmosis (Toxoplasma infection) Toxoplasmosis Frequently Asked Questions*, found at, http://www.cdc.gov/parasites/toxoplasmosis/gen_info/faqs.html. (last visited Nov. 21, 2016).

⁶³⁰ See March 2015 Hearing, at 2–3 (written testimony of Adaora Adimora, M.D.).

⁶³¹ See Jeffrey L. Jones, et al, *Neglected Parasitic Infections in the United States: Toxoplasmosis*, 90 Am. J. Trop. Med. Hyg. 794, 795 (2014).

⁶³² *Id.* at 794.

⁶³³ *Id.*

⁶³⁴ March 2016 Hearing, at 3 (written testimony of Adaora Adimora, M.D.).

⁶³⁵ *Id.* at 3–4.

⁶³⁶ *Id.* at 2 (written testimony of Shannon Weston).

⁶³⁷ See April 2016 Hearing, at 4 (written testimony of Adaora Adimora, M.D.); April 2016 Hearing, at 2 (written testimony of Frederick Askari, M.D.); Schilsky Interview.

⁶³⁸ See March 2015 Hearing, at 4–5 (written testimony of Adaora Adimora, M.D.).

patient advocates expressed outrage that obtaining a critical 60-year-old drug had become such a nightmare.⁶³⁹

The Committee heard about the cases of two infants who were successfully treated for toxoplasmosis only because of the heroic efforts of their physicians.

- At the Committee’s December 2015 hearing, Dr. David Kimberlin, an infectious disease physician at the University of Alabama at Birmingham, told the story of an infant he treated for toxoplasmosis. After Turing acquired Daraprim, the hospital pharmacy could not obtain the drug through Turing’s new limited distribution system, and consequently it was unable to compound it into the liquid formulation infants need.⁶⁴⁰ Further, the 12 months of treatment that infants with toxoplasmosis require, meant that the cost of Daraprim would be prohibitive.⁶⁴¹ While hospital staff were working around-the-clock to find an affordable source of the drug, a small supply purchased before the increase was located on the outpatient community pharmacy shelves, and the infant’s treatment began.⁶⁴² (In general, existing inventory of Daraprim was limited as soon as Turing acquired the drug and sharply raised its price. Internal Turing documents reflect a clear plan to buy up all existing inventory.)⁶⁴³
- In the Committee’s March 2016 hearing, Shannon Weston, the mother of an infant born with congenital toxoplasmosis, shared her family’s story. Her daughter, Isla Weston, born on March 14, 2015, was diagnosed with toxoplasmosis at two months old.⁶⁴⁴ After lab tests confirmed this diagnosis, the infant was prescribed Daraprim, which would need to be taken for about a year.⁶⁴⁵ On the morning treatment was to begin for the infant, Mrs. Weston learned coverage for the medication had been denied by the insurance company.⁶⁴⁶ After a series of re-submissions and appeals, Mrs. Weston was unsuccessful in obtaining coverage. Mrs. Weston described combing websites and exploring all options: “I was hopeless and depressed at the thought of what would happen to my perfect little girl if I was not able to help her. . . . I looked into any way I could think of to come up with the almost \$360,000 necessary to treat my daughter for a year with a drug that she needed, knowing that as long as she was treated before symptoms set in she would remain asymptomatic.”⁶⁴⁷ Finally, help arrived through an unexpected route. The University of North Carolina pharmacy found a source for the active ingredients in

⁶³⁹ See March 2016 Hearing, at 5 (written testimony of Adaora Adimora, M.D.).

⁶⁴⁰ See December 2015 Hearing, at 3 (written testimony of David Kimberlin, M.D.). The infant required a liquid formulation of Daraprim. Ordinarily, to get the liquid formulation, the hospital would acquire the tablet drug, and compound it into a liquid in the hospital pharmacy. *Id.*

⁶⁴¹ *Id.* at 4.

⁶⁴² *Id.* at 4.

⁶⁴³ See *supra*, at 38.

⁶⁴⁴ See March 2016 Hearing, at 1 (written testimony of Shannon Weston).

⁶⁴⁵ *Id.* at 2.

⁶⁴⁶ *Id.* at 2 Mrs. Weston reports in the written testimony, “The morning we were to go for her treatment Dr. Belhorn called to say our insurance company denied covering the medication.” *Id.*

⁶⁴⁷ *Id.* at 2.

Daraprim and was able to compound it onsite, enabling the infant to receive treatment at an affordable cost.⁶⁴⁸

C. Kidney Disease Drug: Thiola

Cystinuria, as mentioned previously, is a rare genetic disease that affects one out of every 10,000 Americans.⁶⁴⁹ Characterized by high concentrations of the amino acid cysteine in the urine, cystinuria leads to persistent kidney stones, the buildup of which is prevented by Thiola.⁶⁵⁰ Without proper treatment, cystinuria causes symptoms such as chronic pain, nausea, and vomiting, and leads to serious damage to the kidneys and surrounding organs. Cystinuria is a chronic disease and treatment lasts a lifetime.⁶⁵¹ While dosage depends on the case and phase of treatment, even maintenance cases typically require taking several pills daily.⁶⁵²

When Retrophin raised the price of Thiola from \$1.50 to \$30.00 per tablet in September 2014, patients and doctors reported difficulty obtaining the drug.⁶⁵³ Concurrent with its price increase, Retrophin moved Thiola to a limited distribution system; a mail order hub controlled by the company.⁶⁵⁴ When this system was first set up, patients reported difficulty accessing and affording the medication, and doctors reported difficulty in completing the additional paperwork to ensure that their patients could obtain the drug.⁶⁵⁵ Dr. Timothy Averch, Director of the University of Pittsburgh School of Medicine Kidney Stone Center, shared that a number of providers got calls from patients who went for routine refills, and were surprised by the significant price increase.⁶⁵⁶ Providers told the Committee that following the initial set-up, they were ultimately able to access the medication for their patients although the process is more arduous due to the additional paperwork.⁶⁵⁷

D. Hospital Administered Cardiac Drugs: Nitropress and Isuprel

Nitropress and Isuprel are cardiac injectable drugs usually administered in hospital settings—often in emergency situations.⁶⁵⁸ Numerous physicians and hospital administrators told the Committee that any patient in their hospitals who truly needs Nitropress or Isuprel

⁶⁴⁸ March 2016 Hearing, at 3 (written testimony of Shannon Weston).

⁶⁴⁹ National Institutes of Health, *Genetics Home Reference* (undated) found at, <https://ghr.nlm.nih.gov/condition/cystinuria> (last visited Dec. 13, 2016).

⁶⁵⁰ See generally, Nicola Sumorok & David S. Goldfarb, *Update on Cystinuria*, 22 *Curr Opin Nephrol Hypertens* 427 (2013).

⁶⁵¹ See Kamran Ahmed et al., *Cystine Calculi: Challenging Group of Stones*, 82 *Postgrad Med. J.* 799, 799 (2006).

⁶⁵² See Thiola Prescribing Information (undated), found at, <http://www.thiola.com/assets/pdf/ThiolaPrescribingInformation.pdf> (last visited Dec. 13, 2016).

⁶⁵³ Committee Staff Interview with Dr. Tim Averch (Mar. 2, 2016).

⁶⁵⁴ See, *supra*, at 43.

⁶⁵⁵ *Id.*

⁶⁵⁶ *Id.*

⁶⁵⁷ *Id.*

⁶⁵⁸ *Id.*

receives it. Many stressed, however, that the price increases imposed by Valeant on these two drugs have placed substantial financial and other burdens on physicians and their institutions.

These price increases affect patients and their families in two ways. First, in an attempt to lower costs, the effort to decrease the use of these drugs to the extent possible required physicians to comb through procedures, identify substitutes, and develop new treatment protocols and associated training.⁶⁵⁹ Physicians reported this led to less time with patients, which affected the care patients receive and added to the inefficiency and cost of the health care system.⁶⁶⁰ Another way many hospitals are rationing their use of these two life-saving cardiac drugs is by not stocking them on every crash cart in the hospital.⁶⁶¹ This cost-saving measure can increase the time it takes a patient to receive these drugs in an emergency, which could have potential adverse clinical repercussions.

Second, because Nitropress and Isuprel remain critical in certain cases, hospitals buying the drugs at exorbitant prices have taken substantial economic hits and had to divert resources from other areas. Dr. Richard Fogel, Chief Clinical Officer of St. Vincent's Hospital in Indiana, testified at the Committee's April 2016 hearing that increased hospital spending on Nitropress and Isuprel would cause the institution to cut back on providing health care services to the broader community served by St. Vincent's. Dr. Fogel cited expansion of the hospital's Rural and Urban Access to Health initiative, which connects low-income and vulnerable communities with health care services, food, transportation, and housing, as well as a number of initiatives to fight the opioid epidemic as casualties of this price increase. The price spikes harm not only patients at the hospital, but also the entire community around the hospital. Dr. Fogel also forecast that continuing increases in the prices of hospital drugs would force some community hospitals to close, causing hardships for the many people they serve.⁶⁶²

1. Burden on Physicians to Find Affordable Drugs or Assistance for Some Patients

In the course of this investigation, the Committee encountered a number of situations where physicians were also affected by the price spikes. For example, physicians told the Committee they have been:

⁶⁵⁹ See April 2016 Hearing, at 4 (written testimony of Richard Fogel M.D.).

⁶⁶⁰ Staff Interview with Bob Rothstein (Johns Hopkins University) (Mar. 24, 2016) ("Rothstein Interview"); Staff Interview with Dr. Richard Fogel (Mar. 24, 2016) ("Fogel Interview").

⁶⁶¹ See December 2015 Hearing, at 3.

⁶⁶² See April 2016 Hearing, at 5–6 (written testimony of Richard Fogel M.D.).

- Required to spend substantial amounts of their time searching for a supply of Daraprim to treat a sick infant;⁶⁶³
- Required to devote time and resources to counsel seniors who are scared they can't afford a life-saving medicine they have been taking for decades;⁶⁶⁴
- Forced to hire extra staff to handle the new burdens of applying and reapplying for insurance coverage that is suddenly denied following a price hike, or to help patients navigate the opaque and byzantine requirements of PAPs;⁶⁶⁵ and
- Forced to design and implement new protocols for conditions calling for hospital drugs that hospitals can no longer afford.⁶⁶⁶

Providers observed that their burden is particularly great when patients are more vulnerable. They help seniors, for example, who face challenges navigating administrative barriers on their own. Some seniors face an additional challenge because they generally do not qualify for co-pay assistance programs, which are prohibited by Medicare. Dr. Michael Schilsky ("Dr. Schilsky"), of the Yale School of Medicine, who treats hundreds of Wilson patients, told Committee staff that 100 percent of his senior patients were having difficulty obtaining their medication.⁶⁶⁷ He described patients hoarding pills, cutting back on doses, and continually facing supply gaps since their coverage generally provided for one month's supply at a time.⁶⁶⁸ Dr. Schilsky said that one result of this unsettling situation is that he spends many hours dealing with insurance companies and PAPs to help his patients get the medications they need.⁶⁶⁹ Similarly, Dr. Jeff Bronstein, a UCLA neurologist, described the requirements of getting needed medicine for many of his patients as a "jungle" to be managed.⁶⁷⁰

2. Burden on Hospitals

Hospital administrators and physicians told the Committee that the dramatic jumps in the prices of Nitropress and Isuprel contributed greatly to the overall rising cost of drugs they were facing. The Ascension Health System, for example, reported a \$12 million budgetary impact in 2015 from these increases, with Nitropress and Isuprel ranking first and second among the hospital drugs that were contributing to its increased costs. The Johns Hopkins Health System reported it suffered a \$1 million hit in 2015 from price increases for Nitropress and Isuprel, and the Cleveland Clinic spent over \$5 million for the two drugs in 2015.⁶⁷¹ The Cleveland Clinic

⁶⁶³ See December 2015 Hearing, at 3 (written testimony of David Kimberlin M.D.).

⁶⁶⁴ See April 2016 Hearing, at 2 (written testimony of Frederick Askari M.D.).

⁶⁶⁵ *Id.*

⁶⁶⁶ See April 2016 Hearing, at 4 (written testimony of Richard Fogel, M.D.); April 2016 Hearing, at 4 (written testimony of Richard Fogel, M.D.); December 2015 Hearing, at 4 (written testimony of Erin Fox, Ph.D.).

⁶⁶⁷ Schilsky Interview.

⁶⁶⁸ *Id.*

⁶⁶⁹ *Id.*

⁶⁷⁰ Bronstein Interview.

⁶⁷¹ See Letter from Scott Knoer to the Hon. Susan M. Collins & the Hon. Claire McCaskill, at 1 (Apr 25, 2016) ("Cleveland Clinic Letter"); Letter from Ronald R. Peterson (Johns Hopkins Health System) to the Hon. Susan M. Collins & the Hon. Claire McCaskill, at 2 (Apr. 25, 2016) ("JHU Letter").

reports that it continues to be adversely affected by the high prices of Nitropress and Isuprel in 2016, despite adopting measures to reduce usage.⁶⁷²

These increased costs put hospital budgets under significant strain. Hospitals generally bill patients for in-patient drugs based on the historical cost of treatment for the hospital's diagnosis-related group ("DRG") rather than billing separately for the drug.⁶⁷³ Updating DRG costs to reflect the increased cost of one component of the treatment would pass the increased cost onto patients and their insurers, but DRG costs are often not updated for a year or more so they don't reflect real-time increases.⁶⁷⁴ Several hospital representatives told the Committee that these price increases have put them in "the red" with respect to coverage for the applicable DRG payments.⁶⁷⁵ This strain on pharmacy budgets can reverberate through a hospital system. Non-profit hospitals, in particular, reported that the price increases led to cuts in different departments, and impinged on programs that help the low-income and vulnerable.⁶⁷⁶ At the April 27th Committee hearing, Dr. Fogel discussed plans that St. Vincent hospital had to create new programs to fight the opioid epidemic, also referenced above, and how the sudden drug price spikes diverted resources from the creation of these new programs.⁶⁷⁷

In the wake of the price increases, hospitals have taken aggressive steps to reduce their usage of Nitropress and Isuprel. For example, Johns Hopkins, the University of Utah, the Cleveland Clinic, and Ascension Health reported taking some or all of these steps:

- Cutting back or eliminating the use of Isuprel on hospital emergency "crash carts."⁶⁷⁸ These are mobile units containing critical supplies that are stationed at various points throughout the hospital for use in emergency situations, such as when a patient suffers a heart attack.
- Physicians told the Committee they have been unable to discharge an HIV patient from the hospital because a supply of the drug could not be obtained to continue the patient's treatment at home, increasing healthcare costs for everyone involved;⁶⁷⁹
- Substituting other drugs where possible. For example, hospitals have been using nicardipine to replace Nitropress in some cardiac procedures and emergencies.⁶⁸⁰

⁶⁷² Katie Thomas, *Valeant Promised Price Breaks on Drugs. Heart Hospitals are Still Waiting*, N.Y. Times, (May 11, 2016).

⁶⁷³ See generally, CMS, *Medicare Claims Processing Manual, Chapter 3—Inpatient Hospital Billing*, Rev. 3504 (Apr. 28, 2016), found at, <https://www.cms.gov/Regulations-and-Guidance/Guidance/Manuals/downloads/clm104c03.pdf> (last visited Dec. 14, 2016).

⁶⁷⁴ See generally, HHS Office of the Inspector General, *Medicare Hospital Prospective Payment System: How DRG Rates are Calculated and Updated*, OEI-09-00-00200 (Aug. 2001).

⁶⁷⁵ Fox Interview; Committee Staff Interview with Cleveland Clinic Pharmacy Department (Apr. 22, 2016); Committee Staff Interview with Lisa Harvey McPherson and James Cattin (Eastern Maine Health System) (Nov. 23, 2015); Committee Staff Interview with Katie Fulham Harris (MaineHealth) (Nov. 23, 2015); Committee Staff Interview with Scott Knoer (Nov. 6, 2015).

⁶⁷⁶ See April 2016 Hearing, at 5 (written testimony of Richard Fogel M.D.).

⁶⁷⁷ *Id.*

⁶⁷⁸ December 2015 Hearing, at 3 (written testimony of Erin Fox, Ph.D.).

⁶⁷⁹ See March 2016 Hearing, at 4 (written testimony of Adaora Adimora, M.D.).

⁶⁸⁰ Rothstein Interview. Nicardipine had been considered expensive relative to Nitropress before the Nitropress price increases, and thus not used in those situations where it might have been.

- Actively looking for alternative approaches.⁶⁸¹
- Aggressively monitoring usage.⁶⁸²
- Reducing inventories.⁶⁸³

Achieving these reductions is itself a costly process for hospitals and staff. A number of physicians and hospital representatives told Committee staff that making the changes is not as simple as substituting a new drug for Nitropress in the hospital pharmacy or issuing new policies.⁶⁸⁴ Administrators must develop new policies and protocols as well as train the medical professionals who treat patients in using them. Many doctors who have used Nitropress and Isuprel for decades must learn new protocols designed to reduce the drugs' use.⁶⁸⁵ Physicians emphasized to the Committee that patients in their hospitals who need one of these cardiac drugs will get them, but they also stressed that transitions are costly. One physician discussed the trial and error factor that implementing new protocols entails, and emphasized that there are no one size fits all replacements for Nitropress and Isuprel.⁶⁸⁶ The increased time that administrators, physicians, nurses, and others who treat patients spend developing policies and learning and implementing new protocols is time away from patient care.

Valeant announced in numerous settings, including in April 2016 testimony before the Aging Committee and in an October 2015 letter to the Committee's Ranking Member, that hospitals across the United States were receiving significant discounts in their purchases of Nitropress and Isuprel.⁶⁸⁷ Valeant officials stated that it was providing volume-based discounts in response to complaints from hospitals and lawmakers to reduce the stress of the sharp price increases on those drugs. According to interviews the Committee conducted with dozens of hospital officials and purchasing organizations, that was not the case.⁶⁸⁸ A number of these hospitals and organizations provided formal submissions for the record prior to the April 2016 Hearing confirming that they had not received—and in many cases had repeatedly sought—such discounts from Valeant.⁶⁸⁹ Only two of the seven GPOs that Committee staff contacted at that time reported having contracts with Valeant that provided for volume discounts. In one case, the predominant discount was one cent off of the WAC—essentially no discount at all.⁶⁹⁰

- Ascension Health, the largest non-profit health care system in the United States, testified in April 2016 that Valeant would not provide any discounts to Ascension, and had denied all requests to contract with the institution.⁶⁹¹

⁶⁸¹ See April 2016 Hearing, at 4 (written testimony of Richard Fogel, M.D.).

⁶⁸² Rothstein Interview; Fogel Interview.

⁶⁸³ *Id.*

⁶⁸⁴ See, e.g., April 2016 Hearing, at 4 (written testimony of Richard Fogel, M.D.).

⁶⁸⁵ Fogel Interview.

⁶⁸⁶ *Id.*

⁶⁸⁷ April 2016 Hearing, at 1–2 (written testimony of J Michael Pearson).

⁶⁸⁸ See, e.g., Cleveland Clinic Letter; JHU Letter; Letter from Erin Fox, Pharm.D. to the Hon. Susan M. Collins & the Hon. Claire McCaskill (Apr 26, 2016) (“Utah Letter”); Submission from Ascension Health (April 2016 Hearing) (Ascension Submission”); JHU Letter.

⁶⁸⁹ *Id.*

⁶⁹⁰ [SEALED] Interviews.

⁶⁹¹ See Ascension Submission, at 1.

- The Johns Hopkins Health System stated that it had neither received discounts nor the offer of discounts from Valeant for in-patient use of Nitropress or Isuprel, and that both drugs remained at their peak price.⁶⁹²
- The Cleveland Clinic reported that it was continuing to pay Valeant’s “exorbitant” prices for the drugs.⁶⁹³
- The University of Utah Health Care system reached out to Valeant in October 2015, and also in March 2016, in an effort to obtain better prices for the two products. Valeant suggested that Utah discuss the prices with its wholesaler, which stated it offered no discounts.⁶⁹⁴
- Aging Committee members shared results of further outreach at the April 2016 Hearing, including Chairman Collins’ statement that a cross-section of Maine hospitals surveyed did not report receiving discounts for Nitropress and Isuprel, and Ranking Member McCaskill’s statement that she had checked hospitals large and small, urban and rural, and the number that reported receiving discounts was zero.⁶⁹⁵

In September 2016, Valeant briefed Committee staff on the status of the company’s commitment, made at the April 2016 hearing, to form a committee to examine options for reducing prices on the four drugs discussed at the hearing: Isuprel, Nitropress, Cuprimine, and Syprine. Valeant officials further briefed Committee staff in September 2016 about the results of that effort.

According to information Valeant provided Committee staff in September briefings, it reached agreements with GPOs under which the bulk of Isuprel and Nitropress would be sold to hospitals at a cost 10 to 15 percent lower than the full prices of \$17,901 per unit for Isuprel and \$881 per unit for Nitropress. Valeant informed staff that it had entered into contracts which included volume discounts with almost all relevant GPOs, with a minimum discount of 10 percent. The information Valeant provided indicated that the vast majority of users would qualify for only a 10 percent discount for Nitropress, and a 10 or 15 percent discount for Isuprel, with a small number receiving larger discounts. Even with these discounts, these drugs remain significantly higher than they were before Valeant began hiking the price of these drugs. At the 10 and 15 percent rebate tiers, Nitropress remains 269 percent higher and 248 percent higher than the original price on the day that Valeant purchased the rights to the drug. At the 10 and 15 percent rebate tiers, Isuprel remains 638 percent higher and 597 percent higher than the original price. Valeant indicated that as of mid-September 2016, there is still one major hospital organization that has not reached a contract agreement with Valeant for these drugs.⁶⁹⁶

⁶⁹² See JHU Letter, at 2.

⁶⁹³ See Cleveland Clinic Letter, at 1–2.

⁶⁹⁴ See Utah Letter, at 1.

⁶⁹⁵ April 2016 Hearing, Trans. at 34:3–35:2, 62:22–63:10.

⁶⁹⁶ See Valeant Responses to Senate Special Committee on Aging (Sept. 13, 2016); Committee Staff Interview with Joseph Papa (Sept 19, 2016).

III. Burden on Government Costs and Insurance Premiums

Rising prescription drug prices affect government budgets as well as private insurers and their individual subscribers. HHS estimated in a March 2016 issue brief that prescription drug spending had risen to \$457 billion in 2015—its highest level.⁶⁹⁷ Expenditures on prescription drugs are projected to continue to rise faster than overall health care spending. Americans cover these expenditures through taxes that fund government programs or directly through commercial insurance plans.

The federal government spends \$126 billion annually on prescription drugs through Medicare, Medicaid, Veterans Affairs, the Children’s Health Insurance Program, and other programs.⁶⁹⁸ Price spikes are contributing to increases in federal government spending. In August 2016, the Centers for Medicare & Medicaid Services released data demonstrating that spending on Medicare Part D drugs increased 17 percent from 2013 to 2014, despite the fact that claims had increased by only three percent.⁶⁹⁹

Insurance companies have also been burdened by sudden price spikes and have sought ways to protect themselves from high prices while providing coverage. In response, they have raised deductibles, increased monthly premiums, transferred high-cost drugs to more expensive tiers, and imposed or increased co-pays. Matt Eyles, executive vice president of policy and regulatory affairs at America’s Health Insurance Plans, a trade organization, is quoted in a *Consumer Reports* article as saying that “the dramatic increase in prescription drug costs is definitely contributing to a move” to increase insurance deductibles and consumer cost share requirements.⁷⁰⁰

⁶⁹⁷ See HHS Office of the Assistant Secretary for Planning and Evaluation, *Issue Brief*, (Mar. 8, 2016), *found at*, <https://aspe.hhs.gov/sites/default/files/pdf/187586/Drugspending.pdf> (last visited Dec. 14, 2016).

⁶⁹⁸ See CMS, *Prescription Drug Expenditures*, National Health Expenditures by Type of Service and Source of Funds, CY 1960-2015, at lines 287, 289, 292, 294, 295, 299, 302, and 308, which totals to \$126.246 billion, *found at*, <https://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/NationalHealthExpendData/NationalHealthAccountsHistorical.html> (last visited Dec. 12, 2016).

⁶⁹⁹ See CMS, *Updated Prescriber-Level Medicare Data*, (Aug. 18, 2016), *found at*, <https://www.cms.gov/Newsroom/MediaReleaseDatabase/Fact-sheets/2016-Fact-sheets-items/2016-08-18.html> (last visited Dec. 6, 2016).

⁷⁰⁰ See Consumer Reports, *Is There a Cure for High Drug Prices?* (July. 29, 2016), *found at*, <http://www.consumerreports.org/drugs/cure-for-high-drug-prices/> (last visited Dec. 14, 2016).

CHAPTER 6. POLICY RESPONSES

The Committee hopes that the sunlight shone on the four companies throughout the investigation will help to deter companies from employing a similar business model and exploiting market failures at the expense of patients. Nevertheless, this troubling practice must be stopped to help rein in price spikes in off-patent, decades-old drugs purchased by companies that did not bear the drugs' research and development costs.

Health economists, physicians, think tanks, and consumer groups have advocated for a wide range of policy solutions from price transparency and controls to compounding and importation.⁷⁰¹ Some experts, however, have cautioned against certain proposals or argued they warrant further study to prevent unintended consequences. The Committee's evaluation of possible policy responses included review of qualitative and quantitative data and current law and practices, consultations with federal agencies, and conversations with numerous experts and stakeholders from all sides of the issues.

The Committee believes there are sound, bipartisan policy solutions that would address the core issues identified by the investigation—market failures that reduce or altogether eliminate competition in decades old, off-patent drugs. With an issue as complex as drug pricing, members understandably have differing views on the merits of the various options available to policymakers, including the responses described in this report. While release of this report does not indicate unanimous support of each of these policy options, we hope that it will contribute to the ongoing discussion.

I. The Increasing Competition in Pharmaceuticals Act

The Committee's investigation has found that older drugs with only one manufacturer and no generic competitor, are more vulnerable to dramatic and sudden price increases.⁷⁰² As a general proposition, generic entry lowers drug prices, with the entry of the second generic having the most downward pressure on pricing.⁷⁰³ It is estimated that on average, generic drugs cost some 80 percent less than brand name drugs.⁷⁰⁴

In March 2016, Chairman Collins and Ranking Member McCaskill introduced *The Increasing Competition in Pharmaceuticals Act* (S. 2615).⁷⁰⁵ This legislation would take steps to incentivize competition and provide solutions to regulatory uncertainty, small market size, and other factors that serve as inherent limitations to generic entry.

⁷⁰¹ See, e.g., Ari B. Friedman, M.D. Ph.D. & Janet Weiner, Ph.D. MPH, *What's the Story with Drug Prices?* Health Policy Sense (May 30, 2016), found at, <http://ldi.upenn.edu/healthpolicysense/what%E2%80%99s-story-drug-prices> (last visited Nov. 29, 2016).

⁷⁰² See, *supra*, at 6.

⁷⁰³ See, e.g., FDA, *About FDA, Generic Competition and Drug Prices*, found at, <http://www.fda.gov/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CDER/ucm129385.htm> (last visited Dec. 14, 2016).

⁷⁰⁴ See, e.g., FDA, *Facts About Generic Drugs* (June 28, 2016), found at, <http://www.fda.gov/Drugs/ResourcesForYou/Consumers/BuyingUsingMedicineSafely/UnderstandingGenericDrugs/ucm167991.htm> (last visited Dec. 14, 2016).

⁷⁰⁵ *The Increasing Competition in Pharmaceuticals Act*, S. 2615, 114th Cong. (2016).

To keep the marketplace competitive, which will help keep drug prices down and improve access for patients, the bill proposes setting a clear timeframe for the FDA to expedite the review of certain generic drug applications to help ensure access to affordable drugs for patients. The bill would also codify the prioritization of first generic products and submissions related to drug shortages (similar to what is referenced in the FDA’s administrative Manual of Policies and Procedures), as well as generic applications for certain older prescription drugs for which patents have expired and for which there is only one manufacturer. These medicines often serve smaller patient populations. This is a critical area that the FDA was failing to prioritize administratively until questioned by Chairman Collins and other Members and following the introduction of S. 2615.⁷⁰⁶ The bill would require the FDA to act to approve or not approve such applications within 150 days. Key provisions of S. 2615 include:

Improving generic access through priority review and timelines. Under the bill, generic applications would be prioritized for review within 150 days for: (1) a drug that has been introduced into interstate commerce by not more than one manufacturer in the last three months and for which there are two or fewer tentative approvals of ANDAs; or (2) a drug that is on the drug shortage list. The GDUFA fees would be waived for most sole-source and drug shortage applications.⁷⁰⁷ Once a drug is off the drug shortage list or there are two manufacturers or more that have introduced the generic in interstate commerce, then any applications submitted do not get priority review or the fee waiver. Under the bill, the Secretary could also expedite the inspection of a manufacturing facility.

Incentivizing companies to enter these important markets by offering a “generic priority review voucher.” The bill would create a new “generic priority review voucher” that would be awarded to the sponsor of a successful application for a medical shortage or sole-source drug subject to priority review under the Act. The FDA would be required to review and act on any ANDA application to which a priority review voucher is applied within 150 days. A voucher would only be awarded to sponsors of approved medical shortage or sole-source drug applications, *not* for those approvals that are already given 180 days of exclusivity as so called “first generics” under existing provisions of Hatch-Waxman. Generic priority review vouchers would be transferable for other generics only. Under the bill, the Secretary could revoke the generic priority review voucher if the generic drug subject to priority review under the bill is not brought to market in 365 days, ensuring that approved applications that receive priority review under the bill are actually marketed. Finally, the voucher program would sunset on October 1, 2022, providing Congress the opportunity to revisit this incentive.

Improve transparency in FDA reporting about generic applications and the backlog. The bill would require the FDA to report to Congress quarterly on the number of ANDA applications filed prior to October 1, 2015, that are still pending; the average and median time such applications have been pending; the number of applications that contain a “paragraph iv”

⁷⁰⁶ See, e.g., *Generic Drug User Fee Amendments: Accelerating Patient Access to Generic Drugs: Hearing Before S. Comm. on Health, Education, Labor and Pensions*, 114th Cong., 2d Sess., Trans. at 28:12–23 (Jan. 28, 2016) (testimony of Janet Woodcock, M.D.) (“January 2016 HELP Hearing”).

⁷⁰⁷ The bill does not waive fees for applications with a “paragraph iv” certification that any patent covering the drug or drug use contained in the ANDA is either invalid or will not be infringed.

certification; and the number that are subject to priority review. This would provide lawmakers with more visibility into the current backlog and help ensure that Congress can perform more oversight of the generic drug review program.

Closing a loophole in the existing priority review voucher program for neglected tropical diseases that Mr. Shkreli tried to exploit. The bill seeks to ensure that a voucher is only granted to a company that did substantial new research, not for research that was done decades ago by another company. While not directly tied to the Committee’s investigation of Turing and Retrophin, Mr. Shkreli tried to exploit such a loophole when he purchased KaloBios Pharmaceuticals, and then quickly attempted to acquire benznidazole, which is used to treat the potentially fatal tropical Chagas disease, from another drug company for \$2 million.⁷⁰⁸ Mr. Shkreli apparently intended to obtain a priority review voucher by filing a topical disease application based almost entirely on decades-old research.⁷⁰⁹

Reporting on the ability of patients, providers, and generic drug companies to access drugs subject to a REMS. Congress authorized the REMS program in 2007 to ensure that sufficient post-market controls could be put in place for the riskiest of drugs to ensure the benefits outweigh the risks of the drug.⁷¹⁰ Since then, concerns have been raised about burdens on providers, pharmacists, and the companies which have to comply with REMS, and concerns that REMS are being used as a barrier to prevent generic access to samples to conduct studies.⁷¹¹ The bill would require, by June 2017, that GAO report to Congress on the REMS program so that Congress has the data and analysis necessary to make decisions about if, or what, changes would be necessary to the program to mitigate any unintended consequences while maintaining the goal of safe and effective drugs.

By authorizing a priority review timeline for generic applications and providing an incentive in certain circumstances, Congress would improve certainty for generic drug companies, help prevent future shortages, increase competition to lower prices and avoid monopolies, and deter practices that can lead to exorbitant price hikes on drugs that were previously affordable for decades.

II. Preventing Generic Entry from Being Blocked

A. Restricted Distribution

Restricted distribution systems are commonplace in the United States; however, changes to the legal regimes governing them are required to ensure that restricted distribution serves its intended purpose. These systems can serve many appropriate ends such as protecting the safety of patients, caregivers, and their families, ensuring patient compliance, and providing

⁷⁰⁸ See Andrew Pollack, *Martin Shkreli’s Latest Plan to Sharply Raise Drug Price Prompts Outcry*, N.Y. Times (Dec. 11, 2015).

⁷⁰⁹ *Id.*

⁷¹⁰ *Food and Drug Administration Amendments Act of 2007*, 121 Stat. 823 (2007).

⁷¹¹ See, *infra*, at 114.

personalized service.⁷¹² As revealed by the Committee’s investigation, these systems can also be abused to delay generic entry in the marketplace. These abuses are serious—by one estimate in 2014, such abuses resulted in an increased cost to consumers of \$5.4 billion per year.⁷¹³

Both Turing and Retrophin (while Mr. Shkreli was CEO) used restricted distribution to try to delay generic entry by preventing potential generic entrants from obtaining the samples of the Reference Listed Drug (“RLD”) needed to complete the bioequivalence testing that is required for FDA approval.⁷¹⁴ Additionally, companies whose drugs are subject to FDA-ordered REMS often use those REMS to defeat or delay generic entry.⁷¹⁵ Two mechanisms can be used to achieve this goal: (1) the restricted distribution component that is common to REMS allows the brand name to block potential generic entrants from obtaining the RLD, and (2) the brand name drug owner can refuse to allow a generic entrant to share the REMS system set up by the brand name for the drug. This blocks generic entry since the REMS regulatory regime requires generics to use the same REMS system as the brand name drug. The FDA has little discretion to waive this requirement, and to date, no brand name company has agreed to terms admitting a generic company into its REMS system.⁷¹⁶

B. Voluntary Restricted Distribution

There are no regulations (outside of REMS) that substantially limit how a company distributes its drugs. Companies are generally free to choose from the varied distribution channels offered by the market, and may voluntarily opt for restricted distribution. In the cases of Turing and Retrophin, placing the drug into restricted distribution was a way for the companies to control who could buy their drugs. Mr. Shkreli blocked any purchase that looked like an attempt by a potential generic entrant to obtain the RLD.⁷¹⁷ To the extent that drugs travelled through less-typical channels (such as 340B institutional distribution), the same rules applied—sales via that channel were carefully regulated and quantity limited to ensure that drugs were not sold to a potential generic entrant.⁷¹⁸

An additional approach is to simply drag out negotiations regarding the sale of the RLD to a potential generic entrant, indefinitely if possible. These extended negotiations often revolve around issues such as non-disclosure agreements, provision of information by the generic entrant to demonstrate that it can conduct safe and effective bioequivalence trials, and allocation of liability regarding use of the RLD by the potential generic entrant in bioequivalence trials.⁷¹⁹

⁷¹² See generally, Lena Y. Choe, Pharm. D., FDA Office of Communication, *Risk Evaluation and Mitigation Strategies (REMS)* (Mar. 25, 2008).

⁷¹³ See *The Creates Act: Ending Regulatory Abuse, Protecting Consumers, and Ensuring Drug Price Competition*: Hearing Before the Subcomm. on Antitrust, Competition Policy, and Consumer Rights, Trans at 19:10–11 (June 21, 2016) (testimony of Ms. Beth Zelnick Kaufman (Amneal Pharmaceuticals)) (“June 2016 Judiciary Hearing”).

⁷¹⁴ See, *supra*, at 36–39, 43–44.

⁷¹⁵ See, e.g., Oral Opinion, at 4–9, *Mylan Pharma v. Celgene Corp.*, No. 2:14-cv-02094 (ES)(MAH) (ECF No. 56) (D.N.J. Dec. 23, 2014) (recounting alleged delay tactics by Celgene).

⁷¹⁶ August FDA Briefing.

⁷¹⁷ See, *supra*, at 38–39.

⁷¹⁸ See, *supra*, at 38.

⁷¹⁹ See, e.g., Oral Opinion, at 4–9, *Mylan Pharma v. Celgene Corp.*, No. 2:14-cv-02094 (ES)(MAH) (ECF No. 56) (D.N.J. Dec. 23, 2014) (recounting alleged delay tactics by Celgene as to drugs on REMS).

Although there is a lack of data in this area, Dr. Woodcock has testified that so called “voluntary REMS” are a major problem:

“[T]he companies on their own behalf have restricted programs that we do not really understand, but they are not related to REMS. We have had over 100 inquiries from generic companies who cannot get a hold of the innovator drug to compare their drug to. We have done everything we can to—we have written a letter saying, you know, that REMS does not require this, you can give it out for this purpose, and so forth, and we also refer these to [the Federal Trade Commission], okay? But we still continue to get complaints from generic companies that they cannot get a hold of the drug to make the comparison they need to do.”⁷²⁰

C. FDA’s Views on REMS

Dr. Woodcock explained, when REMS include elements to ensure safe use, the “REMS program may restrict who gets the drug, right, and that has been used as an excuse or whatever to not give the drug to the generics so they can compare it to their drug.”⁷²¹ This causes “barriers and delays in getting generics on the market.”⁷²² In the typical case, REMS are used in a nuanced manner: the brand manufacturer will not provide the drug because it cannot be sure that a potential generic competitor will handle the drug in accordance with existing REMS and is concerned about attendant liability. The brand company need not refuse to deal with a generic competitor, it may simply engage in never-ending negotiations that have the effect of delaying entry of the generic into the marketplace.⁷²³ The FDA has attempted to stymie this obstruction by providing letters to potential generic entrants indicating that they have reviewed their study protocols and see no safety risk. The FDA’s actions have been largely ineffective to date.⁷²⁴

The second practice centers around the requirement that—ordinarily—a generic competitor share the brand company’s REMS to ensure safe use of the drug. As Dr. Woodcock testified:

“[W]e approve drugs with REMS if they are particularly risky. When they go generic, the generics also need to have this risk system around them. And Congress, in order to decrease the burden on health care, said that if at all possible there be a single shared REMS amongst the innovator and the competitors. Well, this has proven—to get competitors to work together so that the competitors can get a market share from the innovator has proven very challenging for the FDA to get that done, and that has delayed access.”⁷²⁵

⁷²⁰ January 2016 HELP Hearing, Trans. at 51:4–14 (testimony of Dr. Janet Woodcock).

⁷²¹ *Id.* at 50:24–51:2.

⁷²² *Id.* at 51:2–3.

⁷²³ See, e.g., Oral Opinion, at 4–9, *Mylan Pharma v. Celgene Corp.*, No. 2:14-cv-02094 (ES)(MAH) (ECF No. 56) (D.N.J. Dec. 23, 2014) (recounting alleged delay tactics by Celgene).

⁷²⁴ August FDA Briefing.

⁷²⁵ January 2016 HELP Hearing, Trans. 50:13–23 (testimony of Dr. Janet Woodcock).

Typical mechanisms companies use to block access include arguments over indemnity, insurance, non-disclosures, provision of diligence information, and dilatory assertions that portions of the REMS are protected by intellectual property (“IP”) rights or constitute trade secrets.⁷²⁶ In all 13 cases in which the FDA mediated a dispute between generics and brand companies over single shared systems, the FDA ended up authorizing the generic to create a separate REMS.⁷²⁷ The FDA has concluded (setting aside cases of IP or trade secrets) that it only has the power to authorize separate REMS systems if the delay in generic entry has led to a situation where the cost of the drug has affected patient access.⁷²⁸ Accordingly, the FDA will only act after substantial delay. As Dr. Woodcock stated: “Well, the part of the REMS provision that requires a single shared system, as a practical matter, we have to try and try and try and try, and then finally, we declare defeat and we go ahead and let the generics have their own system that is separate but equal.”⁷²⁹

D. Antitrust Law Does Not Provide an Adequate Remedy

Some have suggested that abuses of restricted distribution and REMS appear anticompetitive and therefor violate antitrust laws.⁷³⁰ Further analysis, however, suggests that such abuses do not clearly violate antitrust law and that relying on litigation would not remedy the situation. Legislation and other remedies are needed.

The law is far from clear on whether it is an antitrust violation to refuse to deal with potential generic entrants seeking reference listed drugs.⁷³¹ The conduct of Turing and others, no

⁷²⁶ See, e.g., Oral Opinion, at 4–9, *Mylan Pharma v. Celgene Corp.*, No. 2:14-cv-02094 (ES)(MAH) (ECF No. 56) (D.N.J. Dec. 23, 2014) (recounting alleged delay tactics by Celgene).

⁷²⁷ Committee Staff Briefing with FDA (Aug. 2, 2016).

⁷²⁸ *Id.*

⁷²⁹ Senate HELP Hearing, *Generic Drug User Fee Amendments: Accelerating Patient Access to Generic Drugs*, at 52:2–5 (Jan. 28, 2016) (Testimony of Dr. Janet Woodcock).

⁷³⁰ Antitrust laws include The Sherman Act, 26 Stat. 209 (1890), codified at 15 U.S.C. § 1–7, The Clayton Act, 38 Stat. 730 (1914), codified at 15 U.S.C. §§ 12–27, 29 U.S.C. §§ 52–53, and The Federal Trade Commission Act, 15 U.S.C. §§ 41–58 (as amended.)

⁷³¹ As the U.S. Supreme Court has explained, under antitrust law it is only illegal to willfully acquire or maintain monopoly power via anticompetitive means. By contrast, a monopoly acquired or maintained by virtue of the growth or development of a superior product, business acumen, or even historic accident is not unlawful—nor is charging a monopoly price—so long as it is not accompanied by anticompetitive behavior. The ability to charge short term monopoly prices due to a successful business strategy is important to free markets, and preserves the incentive to innovate. *Verizon Comm’s Inc. v. Trinko*, 540 U.S. 398, 407–08 (2004). Mandating that a business actor share an advantage can lead to anticompetitive results. *Id.* at 408 (noting that “compelling negotiation between competitors may facilitate the supreme evil of antitrust: collusion.”). Accordingly, antitrust law has long held that as a general proposition a business need not agree to deal with its competitors. See, e.g., *Aspen Skiing Co. v. Aspen Highlands Skiing Co.*, 472 U.S. 585, 600–01 (1985). The Supreme Court has been less than clear on when the exception to this rule (*i.e.*, where a refusal to deal is clearly anticompetitive and thus condemned) applies. See generally, *Trinko*, *Aspen Skiing*. Some courts have held that antitrust laws do not require a company to deal unless “a monopolist seeks to terminate a prior (voluntary) course of dealing with a competitor.” *In re Elevator Antitrust Litig.*, 502 F.3d 47, 53 (2d Cir. 2007). Conversely, some courts have held an antitrust action will lie for withholding bioequivalence samples. They reason that, under the controlling Supreme Court authority, refusals to deal are illegal where the defendant has anticompetitive intent evidenced by an election “to forgo . . . short run benefits because it was more interested in reducing competition over the long term.” Oral Opinion, at 11–12, *Mylan Pharma v. Celgene Corp.*, No. 2:14-cv-02094 (ES)(MAH) (ECF No. 56) (D.N.J. Dec. 23, 2014) (internal citation and quotation

matter how disturbing, may be legal.⁷³² Mr. Shkreli and other unscrupulous drug CEOs know this and may have pursued this aspect of the business model precisely because they have precedent supporting the legality of what appears, on the surface, to be anticompetitive conduct. Similarly, brand name manufactures have a strong case that it is legal for them to refuse to admit a potential generic entrant into their single shared REMS system.⁷³³

Additionally, regardless of how the legal question is ultimately decided, it may be a question for the Supreme Court and will take years to resolve. In the Committee's view, patients cannot wait years while companies like Turing drive up prices of decades old, off-patent, and sole-source drugs, by thousands of percentages. Rather, a targeted statutory approach to prevent abuses could be pursued now.

E. Preventing Abuses of Restricted Distribution

Voluntary Restricted Distribution. Non-REMS restricted distribution has led to abuse and there is widespread consensus in the expert community on the solution: create a simple and expedient means for generics to obtain RLDs through a simple and expedited judicial proceeding. The CREATES Act, sponsored by Senate Judiciary Committee Ranking Member Leahy, Chairman Grassley, and Senators Klobuchar and Lee, and cosponsored by Aging Chairman Collins and Ranking Member McCaskill, accomplishes this goal.⁷³⁴ A key provision of the bill would provide a mechanism by which a potential generic entrant can commence expedited litigation to obtain access to samples of the drug needed for bioequivalence studies.⁷³⁵

omitted); see also Oral Opinion, at 114–17, *Actelion Pharma v. Apotex, Inc.*, No. 12-cv-05743 (NLH) (ECF No. 96) (D.N.J. Oct. 17, 2013). Under the view advanced by these cases, termination of a prior course of dealing is powerful evidence of this anticompetitive bent, but is not required. See Memorandum Opinion, at 12–13, *Mylan Pharma v. Celgene Corp.*, No. 2:14-cv-02094 (ES)(MAH) (ECF No. 56) (D.N.J. Dec. 23, 2014); Oral Opinion, at 114–17, *Actelion Pharma v. Apotex, Inc.*, No. 12-cv-05743 (NLH) (ECF No. 96) (D.N.J. Oct. 17, 2013); *Christy Sports LLC v. Deer Valley Resort Co. Ltd.*, 555 F.3d 1188, 1196 (10th Cir. 2009) (voluntary termination of prior course of dealing not required to establish duty to deal).

⁷³² See, e.g., Email from Martin Shkreli to Broadfin Capital, SSCA_THIOL_037833 (Sept. 23, 2014). Because Mr. Shkreli undertook his actions with regards to restricted distribution in New York—within the Second Circuit—*In re Elevator Antitrust Litigation* makes it difficult to bring a successful case against Turing or Mr. Shkreli for failing to deal.

⁷³³ As an initial matter, the very fact that the REMS statute affirmatively proscribes using REMS to block potential generic entrants cuts against an antitrust remedy on the principle that specific statutory provisions take precedence over the general common law of antitrust. *In re Suboxone (Buprenorphine Hydrochloride and Naloxone) Antitrust Litig.*, 64 F. Supp. 2d 665, 688 (E.D. Pen. 2014). The antidiscrimination provision of the REMs statutory scheme has no enforcement mechanism. It plainly lacks rights creating language and certainly does not create an implied right of action. See generally, *Alexander v. Sandoval*, 532 U.S. 275 (2001) (setting forth test for creation of a private right of action). The statute may provide the basis for the FDA to take action against the brand name company, but the lack of a remedial scheme leaves much to debate about the FDA's authority to enforce and consequently little incentive for the FDA to do so. One court held that although a refusal to provide samples for bioequivalency may constitute an antitrust violation because it blocks a generic entrant, failure to allow a competitor to participate in a single shared system of REMs is not an antitrust violation because it does not bar—only delays—FDA approval. *In re Suboxone*, 64 F. Supp. 2d at 688.

⁷³⁴ The CREATES Act, S.3056, 114th Cong. (2016).

⁷³⁵ *Id.*

It would also address some of the concerns raised by brand companies by shielding them from lawsuits predicated on conduct by a potential generic entrant.⁷³⁶

FDA Required REMS. As noted above, the FDA has expressed concern that the current REMS statutory framework is being abused to deter generic entry. Dr. Woodcock of the FDA discussed this problem and a potential solution with Congress:

“Well, the part of the REMS provision that requires a single shared system . . . [i]f that provision were removed from a statute, then potentially, you know, we could just go to that and it would not have a delay involved.”⁷³⁷

The Committee believes that this reform makes sense. The FDA is well-equipped to exercise discretion to allow a potential generic entrant to create its own REMS system while ensuring all applicable safety considerations are met.

III. Reinventing the Federal Trade Commission to Enforce Action

Many commentators, experts, and government officials consulted by the Committee maintain that dramatic price increases in off-patent drugs are the result of unlawful anticompetitive conduct.⁷³⁸ A common theme is that the Federal Trade Commission (“FTC”) needs to exercise more scrutiny in reviewing drug company mergers, operations, and drug market dynamics,⁷³⁹ and that the FTC should be provided with the additional resources and authority it needs to accomplish this goal.

Although antitrust enforcement may seem to be an appropriate tool to combat massive price increases on drugs, it is unclear whether the actions of the four companies examined by the Committee violated current antitrust laws.⁷⁴⁰ The evidence is mixed. It is possible that the business model pursued by the Valeants and Turings of the world was attractive in part because it was legal.

FTC staff has repeatedly suggested that the agency has had to make difficult choices in directing resources, both in merger review and in market oversight.⁷⁴¹ Based on the Committee’s review, the FTC needs more resources to allow it to more vigorously oversee the prescription drug market and carry out its important functions.

⁷³⁶ *Id.*

⁷³⁷ January 2016 HELP Hearing, Trans. at 52:1–8 (testimony of Dr. Janet Woodcock).

⁷³⁸ See Timothy J. Muris, *Everything Old is New Again: Health Care and Competition in the 21st Century*, FTC, 7th Annual Competition in Health Care Forum, at 3, (Nov. 7, 2002) found at, https://www.ftc.gov/sites/default/files/documents/public_statements/everything-old-new-again-health-care-and-competition-21st-century/murishealthcarespeech0211.pdf; Expert Compendium (last visited Dec. 16, 2016).

⁷³⁹ Both the FTC and the Department of Justice have civil jurisdiction to enforce most of the countries antitrust laws. By agreement between the two entities, known colloquially as the “pre-clearance process,” subject matter areas are divided up between the two agencies. Generally speaking, the FTC has jurisdiction of health-care related matters.

⁷⁴⁰ See, *supra*, at 116–17.

⁷⁴¹ Committee Staff Briefing with FTC (Aug. 24, 2016) (“August FTC Briefing”).

The Committee also feels that the FTC should employ additional resources in an effort to better understand the attributes of the drug market. The Committee's findings make clear that the market for prescription drugs is opaque to virtually everyone involved.⁷⁴² In speaking with experts, clarity to regulators would be beneficial.⁷⁴³ Because the FTC was originally enacted not just as an enforcement agency but also as a consumer watchdog, the FTC has broad authority to conduct studies of the marketplace.⁷⁴⁴ The FTC also has power to use compulsory process to this end.⁷⁴⁵ The Committee encourages the FTC to explore a greater use of these powers in the area of off-patent prescription drug pricing.

The Committee also urges the FTC to consider partnerships with academia and other federal agencies. Partnerships with academia present an opportunity for a beneficial symbiosis between the FTC and outside experts that would help provide clarity into drug pricing policies, and whether the market forces that are customarily relied upon to keep prices under control are being thwarted by anticompetitive behavior. This partnership would fill a gap between government agencies and academia, make use of unexploited opportunities, and serve as a model for other agencies.

In health care and drug pricing, the Committee believes there are opportunities for the FTC to work with HHS, DOJ, and the FDA to promote complementary work and harmonization between agencies that will lead to better outcomes for the American people.

Experts also suggested that granting the FTC increased enforcement authority merits further consideration.⁷⁴⁶ While beyond the purview of this report, the Committee agrees.

IV. Temporary Importation During Sudden Price Spikes

One way to combat massive price increases on off-patent drugs would be to allow temporary importation of that drug from countries which follow drug safety standards comparable to those in the U.S. This is a short-term solution favored in some form by a broad spectrum of academics.⁷⁴⁷

The Committee believes that allowing highly-targeted temporary importation to combat major price increases in off-patent drugs could provide prompt price relief. Even so, many experts caution that care must be taken in structuring a temporary importation regime to avoid unintended negative consequences.⁷⁴⁸ The Committee believes that a policy to allow for temporary importation should reflect the following principles:

⁷⁴² Expert Compendium.

⁷⁴³ *Id.*

⁷⁴⁴ Section 6 of the FTC Act, 15 U.S.C. § 46.

⁷⁴⁵ *Id.*

⁷⁴⁶ Expert Compendium.

⁷⁴⁷ See Jeremy A. Greene, M.D., Gerard Anderson, Ph.D., Joshua M. Sharfstein, M.D., *Role of the FDA in Affordability of Off-Patent Pharmaceuticals*, 315 J. of the Am. Medical Assoc. 461, 462 (Jan. 4, 2016); Expert Compendium.

⁷⁴⁸ Expert Compendium.

FDA Authority. The FDA would need to be given additional, express statutory authority to allow temporary importation. The FDA’s existing exercise of regulatory discretion to allow temporary importation (currently only applied in the drug shortages context) has been called into question.⁷⁴⁹ As the D.C. Circuit has explained:

Section 381(a) provides the FDA “shall furnish” to Customs a list of registered establishments and “shall request” from Customs samples of drugs offered for import that are “manufactured, [etc.,] in an establishment not so registered.” Customs, in turn, “shall deliver” to the FDA the requested samples. “If it appears from the examination of such samples or otherwise” that a drug violates a substantive prohibition of the FDCA, then the drug “shall be refused admission.”⁷⁵⁰

Accordingly, the D.C. Circuit held that this language was mandatory and rejected the FDA’s argument that it had discretion to permit importation.⁷⁵¹

Given the D.C. Circuit’s holding in *Cook v. FDA*, the Committee is concerned that any company intent on extracting monopoly rents would seriously consider suing to block temporary importation of drugs⁷⁵² on the grounds that the FDA’s current temporary importation policy is inconsistent with the statute governing importation.⁷⁵³ Accordingly, any policy solution allowing temporary importation should provide the FDA with clear statutory authority in this area.

Limited to Off-Patent Drugs. Importation related to high drug cost (as opposed to shortages) should be limited to off-patent drugs. To allow importation in the case of massive price spikes on drugs subject to patent or statutory exclusivity would undermine the patent and exclusivity system.⁷⁵⁴

Temporary Scope. Any importation would have to be temporary and expire, at the latest, after the anticompetitive practices that led to a functional monopoly in the U.S. had abated. The U.S. drug market is fundamentally different than those abroad. If importation is allowed (even on a narrow list of drugs) without a tightly defined time limit, foreign markets could undercut U.S. markets and likely result in less innovation.⁷⁵⁵ Accordingly, many experts stated the

⁷⁴⁹ To be sure, the text of most statutes imbue agencies with considerable discretion in enforcement (for good reason—they are the experts in highly fact bound areas). See *Chevron USA, Inc. v. Natural Resources Defense Council*, 467 U.S. 837 (1984). It is for this reason that the FDA has discretion not to enforce the general States in the FDA Act governing the use of misbranded or approved drugs. See *Heckler v. Chaney*, 470 U.S. 821, 835 (1985) (refusing to read statute “stat[ing] baldly that any person who violates the Act’s substantive prohibitions ‘shall be imprisoned . . . or fined’” as mandating “criminal prosecution of every violator” of the FDC Act (quoting 21 U.S.C § 333)). But the statute governing importation is different.

⁷⁵⁰ *Cook v. FDA*, 733 F.3d 1, 7 (D.C. Cir. 2013) (citations, quotations, and alternations in original).

⁷⁵¹ *Id.* at 7–12.

⁷⁵² *Cf. id.* at 3–4 (prisoners on death row suing to block importation of sodium thiopental, a drug used in lethal injection protocols, that was made in Austria in a non-FDA approved facility).

⁷⁵³ *Id.* at 9–10.

⁷⁵⁴ Expert Compendium.

⁷⁵⁵ Expert Compendium.

temporary nature of importation should be tightly linked to the market failure that led to monopoly price points.⁷⁵⁶

One potential mechanism would be to only allow importation until an ANDA has been approved. This would temporarily introduce competition into the marketplace while assuring that as soon as there is no longer a monopoly, normal order would be restored. In the view of many experts consulted by the Committee, the importation must be temporary so that it does not discourage entrants from submitting ANDA applications.⁷⁵⁷ If ANDA applicants here or abroad see a faster path to the profits that come to a first generic via importation, they would pursue that course instead.

Preserve Quality Controls. Any approved importation would have to preserve quality controls. While some countries' pharmaceutical manufacturing standards are lax, others, particularly in Canada and the European Union, are stringent and comparable to U.S. standards.⁷⁵⁸ In the context of drug shortages, the FDA allows importation only from those few countries which maintain rigorous regulatory regimes.⁷⁵⁹

Careful Design. Any "trigger" allowing temporary importation must be carefully designed.

Since the FDA's mission is safety and efficacy—not market regulation—any trigger focused on market regulation cannot rest with the agency. The HHS Office of the Assistant Secretary for Planning and Evaluation department of economic analysis, or some other unit suited to analyze market forces at work, would be more appropriate.⁷⁶⁰

The rationale to allow importation must be to remedy a market failure, not to impose price controls.⁷⁶¹ One mechanism to address this would be to link the trigger not to the magnitude of the price, but to the economic impact of the price. Here, the Committee suggests consideration of the so-called "SNIPP" test, a concept drawn from the field of antitrust law. Under this test, the question of whether a company is exercising monopoly power is usually tested by asking whether the hypothetical monopolist could sustain a "small but not insignificant price increase" ("SNIPP") without losing profits. A similar approach could be used here.

V. Copay Coupons and Patient Assistance Programs

Properly structured patient assistance programs can help beneficiaries obtain drugs they might not otherwise be able to afford. Federal regulations allow pharmaceutical companies to contribute funds to third-party PAPs that, in turn, provide copayment assistance to beneficiaries,

⁷⁵⁶ *Id.*

⁷⁵⁷ *Id.*

⁷⁵⁸ FDA, *Global Engagement* (Apr. 30, 2013), *found at*, <http://www.fda.gov/downloads/AboutFDA/ReportsManualsForms/Reports/UCM298578.pdf> (last visited Dec. 6, 2016).

⁷⁵⁹ December 2015 FDA Briefing.

⁷⁶⁰ Expert Compendium.

⁷⁶¹ *Id.*

but only if those third parties operate as independent, *bona fide* charitable entities.⁷⁶² The Committee believes that such programs must not be so narrowly focused that they operate simply as a means by which pharmaceutical companies can subsidize the purchase of their own products.

As previously described, the Committee’s investigation found that self-serving motives were often critical to understanding many patient assistance programs. A key part of Valeant’s strategy was to use “copay cards” to shield privately-insured individuals from the impact of its price increases, enabling the company to sustain its price increases unchallenged (so it assumed).⁷⁶³ Thus, what would seem to be a means to assist patients who are most in need was transformed instead into a mechanism to segment the market to allow the company to maximize its profits from other payers.

The federal Anti-Kick Back Act bars copay coupons in the context of Medicare and other federal health care programs.⁷⁶⁴ Where that prohibition does not apply, however, there is reason to be concerned that companies may use copay assistance to steer patients toward higher priced drugs, resulting in higher expenditures for federal health care programs and commercial providers.⁷⁶⁵

Proponents counter that copay coupons can hold down health care costs by improving patient adherence to treatment regimens, thereby helping to avoid expenses that might otherwise occur, such as hospitalizations.⁷⁶⁶ Some experts recently suggested that prohibiting coupons for cases in which there is a bioequivalent therapy available would provide significant savings.⁷⁶⁷

When there are brand and generic options available for a particular drug, coupons shift spending towards the higher-priced brand drugs, resulting in higher pharmaceutical spending.⁷⁶⁸ Estimates suggest that each copayment coupon increases national spending by \$30 million to \$120 million on average over the five-year period following generic entry.⁷⁶⁹

Additionally, the evidence the Committee obtained suggests that the use of copay coupons have hidden effects on the costs to Medicare beneficiaries and federal taxpayers. For example, while Valeant’s net income attributable to sales of Cuprimine rose from \$14.72 million in 2013 to \$63.52 million in 2015—an increase of 331 percent—the share attributable to sales of the drug to Medicare patients rose at nearly twice that rate, from \$3.38 million in 2013 to \$24.14

⁷⁶² See generally, Supplemental Bulletin updating the OIG Special Advisory Bulletin on Patient Assistance Programs for Medicare Part D Enrollees published in the Federal Register on November 22, 2005 (70 FR 70623), 79 Fed. Reg. 104 (May 30, 2014).

⁷⁶³ See, *supra*, at 19.

⁷⁶⁴ 42 U.S.C. § 1320a-7b(b)

⁷⁶⁵ Congressional Research Service, *Prescription Drug Discount Coupons: Implications for Public and Commercial Health Care Plans*, R44264, at 17 (Nov. 5, 2015).

⁷⁶⁶ *Id.*

⁷⁶⁷ See Leemore S. Dafny, Christopher J. Ody, Matthew A. Schmitt, *Undermining Value-Based Purchasing Lessons from the Pharmaceutical Industry*, 375 The New England J. of Medicine, 2013, 2014 (Oct. 2016).

⁷⁶⁸ *Id.* at 2014.

⁷⁶⁹ *Id.* at 2014.

million in 2015. Over the same two years, net revenues attributable to Cuprimine sales to Medicare patients rose from 23 percent to 38 percent of total Cuprimine net revenues, even while units of Cuprimine sold to Medicare patients held steady at between 22 and 25 percent. This increase was nearly pure profit. While copay coupons appear to help some patients, taxpayers and Medicare beneficiaries are absorbing the increasing costs of exorbitantly priced pharmaceuticals.⁷⁷⁰

VI. Transparency in the Health Care System

While the Committee's investigation was focused on price spikes on specific off-patent drugs, it encountered a phenomenon that is omnipresent in the prescription drug industry—prices are not transparent.⁷⁷¹ Prices are negotiated in closed-door confidential agreements.⁷⁷² Neighboring hospitals may not know what the other is paying. Within the federal government, each agency pays a separate price.⁷⁷³ In the outpatient setting, patients often learn their price by surprise when they visit a pharmacy or receive a bill.⁷⁷⁴ Even researchers shared that the simple question of, “What is the price of a drug?” can be impossible to answer.⁷⁷⁵ Available datasets are imperfect and expensive.⁷⁷⁶

Moreover, the supply chain is not transparent.⁷⁷⁷ The manufacturer is not necessarily the same as the pharmaceutical company with rights over a drug, and neither entity necessarily is the distributor.⁷⁷⁸ On top of these layers of concealed identities, the distribution mechanism is not transparent.⁷⁷⁹ The Committee's investigation uncovered closed distribution systems that further concealed how a drug gets to patients and erected barriers to competitor entry.⁷⁸⁰

The obfuscation continues in that the role of various players along the supply chain is not transparent.⁷⁸¹ PBMs, for example, are tasked with negotiating back and forth between hospitals, employers, and pharmaceutical companies, to reportedly get the best deal.⁷⁸² But it is unclear who the parties are that are getting the best deal as well as how much PBMs charge for

⁷⁷⁰ Figures derived from Email from Brian D. Smith, Esq. to Mark Brian LeDuc, Esq. (Oct. 28, 2016) and Rosiello Interrogatories, at ¶ 8 and attached charts.

⁷⁷¹ Expert Compendium.

⁷⁷² *Id.*

⁷⁷³ CBO Paper, *Prices for Brand-Name Drugs Under Selected Federal Programs*, Congressional Budget Office 4-5 (June 2005).

⁷⁷⁴ Marzolo Interview.

⁷⁷⁵ Expert Compendium.

⁷⁷⁶ IMS dataset costs varied depending on the product and the scope. Costs can vary from thousands to millions of dollars.

⁷⁷⁷ Henry Eichelberg, The American Health Policy Institute, *The Prescription Drug Supply Chain “Black Box”*, at 8 (2015).

⁷⁷⁸ Perry Fry, Health Care Supply Chain Research, *Understanding the Pharmaceutical Supply Chain*, at 5 (July 22, 2015).

⁷⁷⁹ Expert Compendium..

⁷⁸⁰ *See, supra*, at 36–39, 43–44.

⁷⁸¹ *See* Cynthia Challener, *Need for Supply Chain Transparency is Acute*, (Sept. 1, 2014), *found at*, <http://www.pharmtech.com/need-supply-chain-transparency-acute> (last visited Dec. 15, 2016).

⁷⁸² *See* Thomas Gryta, *What is a “Pharmacy Benefit Manager?”*, *The Wall St. J.* (July 21, 2011).

these negotiations. There is good reason to suspect that the patient may not be getting the best deal.⁷⁸³

The funding for health care also lacks transparency.⁷⁸⁴ Chapter Five detailed the role of investors in running pharmaceutical companies' schemes. Companies also fund patient assistance programs to maximize market share.⁷⁸⁵ The source, trajectory, and reach of millions of dollars can get swept under the rug.

This lack of transparency is a serious problem. Markets function best with a robust flow of information, and good policy cannot be implemented without a fulsome record.⁷⁸⁶ The Committee analyzed a range of transparency proposals that would shine light on the health care system. Some experts called for a balanced solution to improving drug pricing transparency in order to avoid unintended consequences.

Reporting AMP After Lag. Transparency in prices would improve transparency in the health care system. Since WAC and AWP prices are not what payers actually pay, many experts have suggested that it would make more sense to report the Average Manufacturer Price ("AMP"), which is based on the price calculated after all sales and rebates are taken into account.⁷⁸⁷ AMP prices are calculated retrospectively; therefore policy proposals to require reporting should factor in a lag period to ensure accuracy of the figure and minimize the potential for that figure to contribute to any market collusion. AMP is the net revenue divided by the number of prescriptions, and is statutorily defined as "the average price paid by wholesalers for drugs distributed to the retail class of trade, net of customary prompt pay discounts."⁷⁸⁸ AMP is well-defined and has been used since 1991. Its calculation is also based on actual sales.⁷⁸⁹

Companies are required to report AMPs for Medicaid; therefore, companies are already calculating these figures.⁷⁹⁰ As discussed above, AMP is a transaction-based price, and it takes several months to calculate AMP because it incorporates sales, discounts, rebates, and relevant pricing information.⁷⁹¹ Releasing AMP data to the public after a lag of 8–12 months would provide the American public with information about how much the drug actually costs payers. This information would empower patients and doctors to make more-informed choices, especially when alternative therapies are available. This proposed disclosure does not claim to solve the problem of price increases, but it would improve transparency in health care and serve as a launching pad for evidence-based negotiation. For example, the AMP figure would serve as

⁷⁸³ Committee Staff Interview with Mark Riley (Nov. 3, 2016).

⁷⁸⁴ See *The Healthcare Imperative: Lowering Costs and Improving Outcomes: Workshop Series Summary*, The Institute of Medicine Roundtable on Evidence-Based Medicine, Washington DC, National Academies Press (2010).

⁷⁸⁵ See, *supra*, at 58.

⁷⁸⁶ See PL Yong, RS Saunders, LA Olsen, *The Healthcare Imperative: Lowering Costs and Improving Outcomes: Workshop Series Summary*, The Institute of Medicine Roundtable on Evidence-Based Medicine, Washington DC, National Academies Press (2010).

⁷⁸⁷ Daniel R. Levinson, *Medicaid Drug Price Comparisons: Average Manufacturer Price to Published Prices*, HHS OIG Report OEI-05-05-00240, 4 (June 2005) (discussing method of AMP calculation); Expert Compendium.

⁷⁸⁸ Daniel R. Levinson, *Medicaid Drug Price Comparisons: Average Manufacturer Price to Published Prices*, HHS OIG Report, OEI-05-05-00240, 4 (June 2005).

⁷⁸⁹ *Id.*

⁷⁹⁰ *Id.*

⁷⁹¹ Expert Compendium.

a reference point for contracts. Hospitals and employers would still negotiate prices; however, unlike the secret negotiations that take place today, AMP disclosure would provide an accurate benchmark. This data would empower payers to negotiate more effectively, and the lag in disclosing it would assuage concerns about anticompetitive behaviors. This data would also be useful to academics who could use it to uncover trends and identify further solutions.

Releasing AMP data after a lag would empower patients and doctors, and prevent surprise costs at the pharmacy or on health bills.

VII. Additional Considerations

In the wake of this investigation, the cost of prescription medications continues to be of great concern to the American public. This investigation touched on but one small segment of the problem: off-patent, sole-sourced prescription drugs. The problem is larger. The news of sudden prices spikes, whether it is EpiPen or insulin or Narcan, is constant. While these problems extend beyond the purview of this investigation and report, the Committee considers further exploration into whether or not the patent system is being abused and the role that supply chain actors may be playing as two areas worthy of further consideration.

Many experts suggested that while the patent system is vitally important to encourage innovations, there are aspects of the system that are being abused.⁷⁹² One area that was frequently mentioned was the use of so called “blocking patents”, which are patents that claim only a minor therapeutic improvement and are more about preventing competitive entry and less about truly advancing the art in question.⁷⁹³

Another area worthy of further study is the role of PBMs. While some experts claim that PBMs function to keep down the price of drugs, other have suggested that PBMs may be contributing to part of the drug pricing problem.⁷⁹⁴ The Committee does not have visibility into this area from its investigation due to the fact that PBMs played a limited role in the drugs investigated by the Committee.

⁷⁹² *Id.*

⁷⁹³ *Id.*

⁷⁹⁴ Committee Staff Interview with Mark Riley (Nov. 3, 2016).

CONCLUSION

The Committee's investigation uncovered disturbing practices in pharmaceutical drug pricing and made substantial progress towards a shared goal of making life-saving medicines affordable and accessible for all Americans. The Committee strongly supports continued efforts to stop bad actors in the pharmaceutical space. Legislative and regulatory changes are required to combat the unwarranted and excessive price hikes that the Committee investigated and that have been so detrimental to patients, providers, hospitals, and taxpayers. Federal policy should strike the right balance between maintaining the incentives needed to promote innovation and the development of new drugs, and keep medicines affordable for patients to support a healthy American public.

GLOSSARY

Term	Definition
Abbreviated New Drug Application (ANDA)	Submitted by the sponsor to market a generic of a drug product in the United States. It contains data to show that a generic drug product is bioequivalent to the reference listed drug and comparable in dosage form, strength, route of administration, quality, performance characteristics, and intended use.
Actual Acquisition Cost (AAC)	Final cost of drugs to pharmacy after all discounts, rebates, and price concessions (not defined in statute or regulations).
Average Manufacturer Price (AMP)	AMP for a covered outpatient drug for a rebate period is the average price paid to the manufacturer for the drug in the United States by (i) wholesalers for drugs distributed to Retail Community Pharmacies (RCPs); and (ii) RCPs that purchase drugs directly from drug manufacturers (SSA §1927(k)(1)).
Average Selling Price (ASP)	The average price at which the drug is sold across channels or markets.
Active Pharmaceutical Ingredient (API)	The ingredient in the pharmaceutical product which is biologically active.
Authorized Generic	A brand-name prescription drug that is sold, marketed, and distributed as a generic under a private label company.
Average Wholesale Price (AWP)	Commercially published reference price but not an average price paid by purchasers or charged by wholesalers. AWP is considered a manufacturer's suggested wholesale price to the retailer as listed in published drug industry compendia (not defined in statute or regulations). The AWP is set by manufacturers and used by Medicare to set reimbursement rates.
Bioequivalent	The biological equivalence of two proprietary preparations of a drug including its use, efficacy, safety, dosage form, route of administration, strength, and active substances. Drugs that are bioequivalent are expected to perform in the same manner within a comparable biological systems.
Brand Name Drug	Brand name drugs are those drugs sold under an applicable brand name (<i>e.g.</i> , Daraprim). It is possible to have both innovator Source Drugs that are branded, and generics that are branded (known as authorized generics).
Best Price	Best price for single source and innovator multiple source drugs is the lowest price available from a manufacturer during the rebate period to any U.S. entity in any pricing structure (including capitated payments) for the same quarter as the AMP is reported (42 CFR §447.505).
Compounding	The process in which a pharmacist combines, mixes, or alters various ingredients to create a medication that is “tailored to the needs” of an

	individual patient. Compounding is generally used to prepare medications that are not commercially available, such as a drug in a lower dosage for a child, or a drug without a dye or a preservative in response to a patient allergy.
Consumer Price Index—Urban (CPI-U)	CPI-U is the index of consumer prices developed and updated by the U.S. Department of Labor. It is the CPI for all urban consumers (U.S. average) for the month before the beginning of the calendar quarter for which the rebate is paid (42 CFR §407.502).
Estimated Acquisition Cost (EAC)	EAC is a Medicaid agency’s best estimate of the price generally and currently paid by providers for a drug marketed or sold by a particular manufacturer or labeler in the package size of drug most frequently purchased by providers (42 CFR §407.502).
Generic Drug	Non-innovator drugs that are bioequivalent to respective brand name drugs. The term generic drug is not defined in statute (OIG).
Generic Drug User Fee Act (GDUFA)	The Generic Drug User Fee Amendments (GDUFA) established a user fee program for generic drugs (effective October 1, 2012, - October 1, 2017). The purpose is to maintain U.S. quality standards for generic entrants while increasing the likelihood of timely approvals.
Gold Standard Drug	The gold standard drug is considered by experts in the field to be the best drug available for the condition it treats.
Group Purchasing Organization (GPOs)	Group purchasing organizations negotiate contracts with wholesalers or manufacturers on behalf of a number of hospitals or other providers.
Hatch-Waxman Act	Drug Price Competition and Patent Term Restoration Act of 1984 (P.L. 98-417), commonly known as the Hatch-Waxman Act, made significant changes to the patent laws as they apply to pharmaceutical products in an attempt to balance the need for innovative new drugs and the availability of less expensive generic products. The act created practices intended to facilitate the marketing of generic drugs while permitting brand name companies to recover a portion of their intellectual property rights lost during the pharmaceutical approval process.
Innovator Multiple Source Drug	A drug that was originally marketed under an original NDA approved by the Food and Drug Administration (“FDA”), including an authorized generic drug. It includes a drug product marketed by any cross-licensed producers, labelers, or distributors operating under the NDA and a covered outpatient drug approved under a product license approval (PLA), establishment license approval (ELA), or antibiotic drug approval (ADA) (42 CFR §407.502).
Monopoly Rent	An economic term meaning profits above those that would be available in a competitive market, received by a supplier in a market where supply is artificially restricted.
Multiple Source Brand Name Drugs	Drugs that are available from a brand name manufacturer and also have generic equivalents.

National Drug Acquisition Cost (NADAC)	The national price benchmark of the costs that pharmacies pay to acquire prescription and OTC drugs, based on invoice cost data collected from pharmacies that reflect actual drug purchases (CMS, Draft Methodology for Calculating the National Average Drug Acquisition Cost, Part II, May 2012).
National Drug Codes (NDCs)	Unique codes consisting of 10-digits broken up into three segments to identify each drug by the marketing pharmaceutical company, drug strength, and package size. Medicaid uses NDCs identify unique formulations of each drug(OIG).
New Drug Application (NDA)	The process through which drug sponsors propose that the FDA approve a new pharmaceutical for sale and marketing in the U.S.
Non-innovator Multiple Source Drug	Also known as generic drugs and are defined as (1) a multiple source drug that is not an innovator multiple source drug or a single source drug, (2) a multiple source drug that is marketed under an abbreviated NDA or an abbreviated antibiotic drug application, or (3) a drug that entered the market before 1962 that was not initially marketed under an original NDA (42 CFR §407.502).
Off-Patent Drug	Any drug that is not the subject of an unexpired patent or exclusivity period.
Orange Book	The publication Approved Drug Products with Therapeutic Equivalence Evaluations identifies drug products approved on the basis of safety and effectiveness by the FDA under the Federal Food, Drug, and Cosmetic Act. Patent listings can be found in this online book, which is updated daily.
Red Book	The Red Book lists drugs and their prices. In general, the Red Book lists the average wholesale price (AWP) of drugs. The price list is updated quarterly.
Reference Listed Drug (RLD)	A reference listed drug is an approved drug to which new generic versions are compared to demonstrate bioequivalence.
Retail Community Pharmacy (RCP)	Retail community pharmacies are state-licensed independent pharmacies, chain pharmacies, supermarket pharmacies, or mass merchandiser pharmacies that dispense medications to the general public at retail prices. RCPs do not include pharmacies that dispense prescription medications to patients primarily through the mail-order, nursing home pharmacies, long-term care facility pharmacies, hospital pharmacies, clinics, charitable or not-for-profit pharmacies, government pharmacies, or PBMs (SSA §1927(k)(10)).
Risk Evaluation and Mitigation Strategies (REMS)	Risk Evaluation and Mitigation Strategies (REMS) were authorized by The FDA Amendments of 2007 to ensure that the benefits of a drug or biological product outweigh its risks. The FDA can require a REMS before or after a drug is approved. Each REMS has specific safety measures unique to the safety risks associated with the particular drug.
Patient Assistance Programs (PAPs)	Offered by pharmaceutical manufacturers to help indigent, uninsured, or underinsured patients obtain the prescription drugs they need. By statute, individuals covered by government insurance are not eligible for PAPs.

Pharmacy Benefit Managers (PBMs)	Pharmacy benefit managers (PBMs) act as intermediaries between health care payers and other players including drug manufacturers, pharmacies, and employers.
Sole-Source Drug	A sole-source drug is a covered outpatient drug produced or distributed under an original NDA approved by the FDA, including a drug product marketed by any cross-licensed producers or distributors operating under the NDA. It also includes a covered outpatient drug approved under a biological license application, PLA, ELA, or ADA (42 CFR §407.502).
Sherman Act	The Sherman Anti-Trust Act (26 Stat. 209, UF U.S.C. §§ 1-7) approved on July 2, 1890, prohibits business practices that federal government regulators deem to be anticompetitive, and requires the federal government to investigate trusts.
Wholesale Acquisition Cost (WAC)	The manufacturer's list price for the drug or biological to wholesalers or direct purchasers in the United States, not including prompt pay or other discounts, rebates or reductions in price, for the most recent month for which the information is available, as reported in wholesale price guides or other publications of drug or biological pricing data (SSA §1847A(c)(6)).
Wholesaler	A drug wholesaler is engaged in wholesale distribution of prescription drugs to retail community pharmacies, including manufacturers, re-packagers, distributors, own-label distributors, private-label distributors, jobbers, brokers, warehouses (including manufacturer's and distributor's warehouses, chain drug warehouses, and wholesale drug warehouses), independent wholesale drug traders, and retail community pharmacies that conduct wholesale distributions (SSA §1927(k)(11)).
340B	The 340B Drug Discount Program is a U.S. federal government program created in 1992, in section 340B of the Public Health Service Act, requiring drug manufacturers to provide outpatient drugs to eligible health care centers, clinics, and hospitals (termed "covered entities") at reduced costs.