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BEFORE THE SENATE JUDICIARY COMMITTEE

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Chairwoman Klobuchar, Ranking Member Lee, and Members of the Committee, thank you for inviting me to participate in today's hearing. The robust competition that exists in today's biopharmaceutical marketplace is critical to both innovation and affordability, and I appreciate the opportunity to explore this topic with you in depth.

I am here today on behalf of the Pharmaceutical Research and Manufacturers of America (PhRMA). PhRMA represents the country's leading innovative biopharmaceutical research companies, which are devoted to discovering and developing medicines that enable patients to live longer, healthier, and more productive lives. The biopharmaceutical sector is one of the most research-intensive industries in the U.S. Since 2000, PhRMA member companies have invested more than $900 billion in the search for new treatments and cures. In 2019 alone, PhRMA member companies invested $83 billion in research and development (R&D), the highest level of investment on record.[1]

The incredible progress that the biopharmaceutical industry has made in the fight against COVID-19 is a reflection of the decades of private-sector investments in infectious disease, deep scientific and technical expertise, and strong intellectual property protections that have supported an unprecedented level of R&D investment as well as new levels of collaboration and coordination to get as many shots in arms around the globe as possible.

PhRMA appreciates the Committee's interest in opportunities to enhance competition in the health care marketplace. It is more critical now than ever that we advance thoughtful policies that continue to incentivize critically needed innovation while supporting a competitive marketplace. We support market-based solutions that will spur continued brand-to-brand, generic, and biosimilar competition while incentivizing medical advances critical to saving and improving patient lives. My comments provide context on the role of medicines in improving health outcomes for patients; the statutory frameworks that increase access to generic and biosimilar medicines while preserving incentives for innovation; antitrust remedies; the competitive marketplace for prescription medicines; the role and importance of IP rights to support innovation and foster competition through public disclosure of inventions; and several areas of market distortions in the health care marketplace.

At the outset, it's important to recognize how well competition has worked in the biopharmaceutical marketplace. Competition has shifted utilization from brand medicines to generics so successfully that 91 percent of all prescriptions for drugs are in the U.S. are currently filled with generic medicines. Robust brand to brand competition as well as competition from generics and biosimilars and other factors has resulted in prescription medicines accounting for just 14 percent of total health care spending over the past decade, even while many new, breakthrough treatments entered the market to help patients who previously had no therapeutic options.[2] In fact, net per capita spending on prescription medicines has remained effectively flat, increasing just 0.5 percent on average per year over the past 10 years.[3] On average, brand medicine net prices have increased in line with or below

the rate of inflation for the past five years; and last year, net prices for brand medicines *declined* by 2.9 percent on average. [4]

Prescription Medicines are Transforming the Trajectory of Disease

Prescription medicines play a central role in transforming the trajectory of many debilitating diseases, resulting in decreased death rates, improved health outcomes, and better quality of life for patients.

- *Cardiovascular disease:* Tremendous strides have been made against cardiovascular disease over the past 40 years, due in large part to advances in treatment. Since 1980 alone, the death rate from heart disease has declined by more than 50 percent.[5] And between 1980 and 2000, approximately two-thirds of the decline in coronary heart disease mortality, the most common type of heart disease, is attributable to medical therapies.[6]

- *HIV/AIDS*: Once considered acutely fatal, HIV/AIDS is now a chronic and manageable disease. This dramatic change followed the introduction of highly effective antiretroviral therapy in the mid-1990s, which transformed treatment and led to a 91 percent decline in death rates in the United States.[7] Between 2010 and 2017 alone, the death rate from HIV has declined by nearly half. Much of this decline CDC attributes to improvements in early diagnosis and helping people get on and stay on lifesaving treatment.[8]

- *Hepatitis C:* More recently, we've seen a remarkable transformation in treatment of another viral disease: hepatitis C. Just ten years ago, the only available treatment cured just half of patients and caused debilitating side effects. Today, a broad range of treatments with increasing efficacy and minimal side effects and cure rates approaching nearly 100 percent are available for patients with all forms of the disease, including many challenging to treat subpopulations.[9,10] The introduction of curative medicines also reduces health care costs previously associated with treated hepatitis C. In Medicaid, these medicines have been estimated to produce a total of $12 billion in savings net of treatment costs by 2022.[11]

- *Cancer:* New medicines are also driving gains in the life expectancy of cancer patients. Since peaking in the early 1990s, cancer death rates in the United States have declined 31 percent.[12] Researchers attribute 73 percent of these gains to new treatments, including new medicines.[13] Targeted therapies and emerging immunotherapies are transforming the treatment paradigm for patients with many forms of cancer and have the potential to reduce the use of traditional forms of cancer treatment—including chemotherapy, surgery, and radiation.[14] As a result of remarkable advances, between 2000 and 2016 alone, new cancer drug approvals in the U.S. have been associated with 1.3 million avoided cancer deaths across 15 of the most common tumor types.[15]

Researchers are pursuing cutting-edge research and novel scientific strategies to continue to drive therapeutic advances for patients. There are currently more than 8,000 medicines in clinical development globally with the potential to impact U.S. patients.[16] And across the medicines in the pipeline, 74 percent have the potential to be first-in-class treatments.[17] Medicines in development include:

- *Cell and Gene Therapies*: Cell and gene therapy represent overlapping fields of biomedical research with similar therapeutic goals, which target DNA or RNA inside or outside of the body. Both approaches seek to modify genetic material to improve functioning or fight disease. Cell therapy involves taking cells from the patient or a donor and genetically altering and reinserting into the patient to treat the underlying cause of the disease. Gene therapy treatment involves making an addition to, silencing, or altering a gene. These treatments represent the translation of basic scientific understanding into innovative new treatment options for patients. There are nearly 400 cell and gene therapies in development focused on a variety of diseases and genetic conditions, ranging from blood disorders, eye disorders, cancer, and infectious diseases, among others.[18]

- *Cancer*: In addition to the cell and gene therapy approaches that are just beginning to transform the lives of patients, several novel approaches – including, antibody-drug conjugates, immune checkpoint modulators, personalized medicines, RNA therapeutics, metabolic immunotherapies, and vaccines – are showing tremendous promise across the pipeline against a broad range of cancers. Today, there are more than 1,300 medicines and vaccines currently in development for cancer.[19]

- *Diseases Affecting Children:* New treatment options for infants, children and adolescents can be complex and often require different clinical approaches than adult treatment pathways. There are currently nearly 600 pediatric medicines currently in development to meet the unique needs of children. Potential medicines include treatments for pediatric patients with range of conditions like genetic diseases, many forms of cancer, infectious diseases, and skin disorders. [20]

Today's biopharmaceutical pipeline has tremendous promise and represents a new frontier of research with the potential to continue to transform the lives of patients. In this new era of medicine, science that was once considered unimaginable is now on the verge of producing a complete paradigm shift in the treatment of the most complex and challenging diseases of our time. As the health care market continues to evolve towards value-driven payment and greater patient engagement in health care decision-making, we need to ensure it is sustainable and balances patient access to innovative medicines without sacrificing investment in further treatments and cures.

Overview of the Statutory Frameworks that Increase Competition while Preserving Incentives for Innovation

As noted by former Director of the U.S. Patent and Trademark Office (PTO) Andrei Iancu, "the progress we have made in the past 200 years is absolutely unparalleled in human history and most of that has been backed by patents."[21] That progress is due to recognition by the Framers of our Constitution of the importance of robust IP protections, empowering Congress in Article 1 Section 8 of the Constitution "To promote the progress of science and useful arts, by securing for limited times to authors and inventors the exclusive right to their respective writings and discoveries." Under Section 101 of the Patent Act, 35 U.S.C. § 101, Congress provided that broad categories of inventions are eligible for patent protection: new and useful processes, machines, manufactures, or compositions of matter, as well as "any new and useful improvement."

In the biopharmaceutical sector, Congress recognized the need to provide approval pathways that foster competition through the market entry of generic and biosimilar medicines while also maintaining incentives for innovation. Two key statutory frameworks simultaneously reward innovation while establishing streamlined approval pathways for generic or biosimilar products. Both patents and the exclusivities provided under the statutory schemes, the Drug Price Competition and Patent Term Restoration Act of 1984, also known as the Hatch-Waxman Act, and the Biologics Price Competition and Innovation Act (BPCIA), have been successful in both fostering innovation and creating robust generic and growing biosimilar markets.

Toward that end, the Hatch-Waxman Act strikes a careful balance between innovation and access. Hatch-Waxman was enacted in response to a landscape in which innovator companies were losing substantial effective patent life during clinical development and the FDA review and regulatory approval process, while at the same time generic companies lacked a reliable abbreviated pathway for approval of generic copies of drugs approved after the 1962 amendments to the Federal Food, Drug, and Cosmetic Act once IP protections expired. The Hatch-Waxman Act created a framework that allowed generic companies to develop products during the period of innovator patent protection without liability for patent infringement,[22] overturning a Federal Circuit decision to the contrary,[23] and seek FDA approval to market products immediately upon patent expiration, or even prior to patent expiration if they successfully challenge patents through the litigation framework created by the Hatch-Waxman Act. Given the nature of the framework created, patent litigation is a natural part of the generic pathway, as are settlements of such litigation. The patent challenge procedure under the Hatch-Waxman Act has proven to be a robust means for generic applicants to seek to market generic versions prior to expiration of listed patents. As a result, the effective patent life for small molecule medicines is about 12 years, meaning brand medicines typically face generic

competition at between 12 and 13 years after brand launch even though the basic patent term is 20 years.[24] Over the 37 years since enactment of the Hatch-Waxman Act, patent challenges from generic manufacturers (in the form of paragraph IV certifications) have been filed more frequently and earlier in the brand-name drug life cycle, with many patent challenges coming at the earliest time possible under the statute—in the case of a new chemical entity, as early as 4 years after FDA approval.[25]

These frameworks have fostered competition in the biopharmaceutical marketplace. For example:

- Prescription drug costs have remained a small and stable share of health care spending year after year because our market-based system leverages competition to control costs throughout the lifespan of a prescription medicine. [26]

- Over the past 10 years, net per capita spending on medicines has remained effectively flat, increasing just 0.5 percent, on average, per year.[27]

- While patents might prevent a competitor from bringing an exact duplicate of a medicine to market during the term of the patent, they do not act as an absolute bar against bringing similar, but non-infringing, products to market. For example, in less than a year after market entry of the first breakthrough treatment for hepatitis C, from a class of treatments known as direct-acting antivirals (DAAs), multiple competitors entered the market, resulting in lower prices and improved cure rates for patients. The competition was so fierce that the average net daily cost for the range of medicines available in the DAA class today is nearly 80 percent lower than the launch price of the first breakthrough DAA.[28] In the case of the cholesterol-lowering drugs, PCSK9 Inhibitors, large purchasers have used their market power to negotiate significant rebates and discounts. Today, net prices are 78 percent lower than the list prices of the first to market PCSK9 inhibitor to enter the market.[29]

- The competitive market is structured to take maximum advantage of savings from brand competition. Competition among brand medicines often begins well before approval, as companies race to be first to market. Multiple companies simultaneously compete to research, develop, and secure FDA approval of first-in-class treatments. In fact, 88 percent of first-in-class medicines launched between 2005 and 2011 already had a competitor in Phase II clinical development at the time of their launch. For drugs approved between 2005 and 2011, the average time an approved medicine was alone in its class was 2.3 years.[30]

- Following generic entry, the U.S. market continues to drive long-term affordability by taking maximum advantage of the savings provided by generic drugs. Today, more than 90 percent of all prescriptions for drugs in the U.S. are filled with generics—due largely to the concentration of purchasing power by payers and the aggressive use of utilization management tools to rapidly shift utilization towards generics.[31] Competitive pressure resulting from the expiration of IP protection is expected to fuel this dynamic in the years ahead, with competition from generics and biosimilars expected to reduce U.S. brand sales by $128 billion through 2025.[32]

The BPCIA, enacted in 2010, was intended to strike a balance between providing access to biosimilar medicines and preserving incentives for innovation in the biologics space. Through the BPCIA, Congress created an abbreviated approval pathway for biosimilar and interchangeable biological products. Biosimilar applicants also may develop products during the period of innovator patent protection without liability for patent infringement. At the same time, Congress provided incentives for innovation by providing for a data protection period governing when biosimilar applications could be submitted (as early as four years after approval) and approved (as early as 12 years after approval of the reference or innovator product). Congress also created a different procedure for litigating in court validity and applicability of patents covering the biosimilar product. Although the dynamics of the Hatch-

Waxman Act and BPCIA litigation procedures differ, they both allow for, and naturally lead to, premarket patent litigation.

While the BPCIA is a little more than a decade old, and biosimilar development is significantly more complex and expensive than generic drug development, the benefits of the BPCIA for innovation and competition are already coming to fruition. The FDA approved the first biosimilar product for marketing in the U.S. in March 2015 and, as of July 2021, 29 biosimilars have been approved in the U.S, with 20 already available on the market. Recognizing that the European Union (EU) has had a biosimilars pathway in place for a longer time period, the U.S. biosimilar approval rate is comparable to the EU at the same point in time.

Several factors have been cited as potentially impacting the timing of biosimilar market entry and success of biosimilars, including costs, complexities related to manufacturing and scale up of manufacturing, and lack of awareness and education among potential prescribers and payers of biosimilars.

FDA is implementing an action plan aimed at accelerating the market entry and uptake of these extremely complex products, which are in many ways more difficult to develop and produce than small molecule pharmaceuticals. PhRMA supports the FDA's efforts to implement a science-based regulatory approach in a timely manner that will ensure patient safety while facilitating a robust biosimilars market. We concur with the FDA that physician education and experience with biosimilars will be critical to fostering biosimilar uptake, as it was for generic drugs. We support FDA's continued efforts to develop "effective communications to improve understanding of biosimilars among patients, clinicians, and payors."[33] These efforts have raised awareness of the FDA's role in the biosimilar approval process, increasing the public's understanding of both biologics and biosimilars, and helping stakeholders understand the data and information that inform biosimilarity determinations. Congress has also recently taken action to help increase understanding. In April 2021, the Advancing Education on Biosimilars Act was signed into law,[34] encouraging FDA to maintain and operate an educational website and advance awareness among health care providers, patients and caregivers about biosimilar and interchangeable biosimilar biological products.

We agree that confidence among stakeholders is essential to developing a robust marketplace for biosimilar products. As America's health care system continues to evolve, biosimilars will play an increasingly critical role in bringing new options to patients and decreasing prescription drug spending. Annualized savings from biosimilars reached $6.5 billion in the second quarter of 2020, and savings are modeled to exceed $100 billion in aggregate over the next five years.[35]

IP fosters both innovation and competition, and these dual purposes can be enhanced with carefully crafted statutory schemes. The Hatch-Waxman Act and the BPCIA are two such schemes, with the Hatch-Waxman Act creating today's robust generic marketplace and the BPCIA well on its way to expanding competition from biosimilars in the biologics marketplace.

Existing Antitrust Laws and Authority Effectively Support Competition

Current antitrust laws, as well as the authority to enforce those laws, have played a critical role in addressing allegations of anticompetitive conduct on a case-by-case basis. The three core U.S. antitrust laws, the Sherman Act, the Federal Trade Commission (FTC) Act, and the Clayton Act prohibit business practices and mergers that unreasonably deprive consumers of the benefits of competition, resulting in higher prices for products and services. These antitrust laws do not regulate pricing itself but focus instead on anticompetitive behavior that excludes competition in a manner harmful to consumers. The Sherman Act, which addresses agreements that unreasonably restrain trade (Section 1) and unilateral conduct like unlawful monopolization (Section 2), carries severe civil and criminal penalties and is enforced by the Department of Justice as well as private litigants. The FTC Act generally prohibits "unfair methods of competition" and "deceptive acts or practices" and captures conduct not covered by the

Sherman Act, though it is well-recognized that violations of the Sherman Act also violate the FTC Act. However, only the FTC can enforce Section 5 of the FTC Act, which specifically prohibits unfair methods of competition.

Thus, price increases or reductions in output that result from anticompetitive behavior—collusive conduct, such as price fixing and bid rigging, as well as abuses of market power, such as anticompetitive tying and exclusive dealing—are already within the scope of existing competition law. Likewise, there are existing legal frameworks to address allegations of sham litigation, fraud on the FDA, and fraud on the patent office.

Policy approaches that seek to address perceived abuses of the patent system should distinguish customary business behavior and rigorous competition from any clearly identifiable conduct that interferes with the competitive process, whether via agreement or unilateral conduct. It would be inappropriate to put FTC in the role of substituting its business judgment for that of companies and second-guessing companies on a retrospective basis, which could have a substantial chilling effect on innovation or even punish procompetitive behavior. The current antitrust laws as well as the authority to enforce those laws are generally sufficient to address allegations of anticompetitive conduct on a case-by-case basis.

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

Medicines have revolutionized the treatment of many serious health conditions, saving lives, improving quality of life, and reducing the need for hospitalization. Prescription medicines have also been shown to be powerful tools to reduce overall health care costs for many conditions. In fact, a recent *Health Affairs* article concluded that one-half of the spending slowdown among Medicare beneficiaries between 1999 and 2012 was attributable to slower growth in spending for cardiovascular diseases; and of this savings, one-half was attributable to use of medications to treat cardiovascular risk factors.[36]

Looking forward, continued advances and better use of medicines will be indispensable in addressing some of our society's biggest health and economic challenges. Research shows better use of medicines, such as improved adherence to needed treatments, would save an estimated $213 billion per year in avoided health care spending.[37] As medicines' role in effective health care has grown sharply, and many new medicines have been brought to patients, retail and physician-administered prescription medicines combined have remained at just 14 percent of total U.S. health care spending.[38] New medicines developed by biopharmaceutical innovator companies accounted for less than one-half of all spending on prescription medicines—or about 7 percent of total health care spending in 2018.[39]

The ability to bring important medical advances to patients while holding medicines' share of health spending nearly constant is made possible by the highly competitive structure of the U.S. market. Fierce market competition among brand, generic, and biosimilar medicines shifts utilization from brand medicines to generics and biosimilars and results in sizable rebates, discounts, and other price concessions. As a result, net prices for brand medicines are, on average, 44 percent lower than the list price.[40] In 2018, net prices for brand-name medicines declined by 2.9% after discounts and rebates were paid, continuing a downward trend for the past five years that was interrupted in 2019.[41] And brand medicine net prices have grown in line with or below the rate of inflation for the past five years.[42] This trend of flat or declining net medicine prices low growth is expected to continue; IQVIA projects annual net price growth for brand-name drugs will be just 0 to -3 percent through 2025.[43]

The competitive market with appropriate IP protections is the engine that drives the innovative biopharmaceutical R&D ecosystem. The dynamics of the private, market-based system in the U.S. promote incentives for continued innovation and increased patient access to needed medicines while leveraging competition to achieve cost containment.

The U.S. market is structured to take maximum advantage of savings from competition while ensuring Americans have access to innovative and life-saving treatments. Today, the U.S. is the global leader in R&D related to lifesaving

treatments and cures. There are nearly 8,000 medicines in development globally, more than half of which are in development in the U.S., including hundreds for conditions like cancer and Alzheimer's disease.[44] The U.S. develops more new medicines than the rest of the world combined,[45] precisely because we reject government price setting and protect IP.

As a result, the U.S. biopharmaceutical sector serves as one of the biggest employers and investors in U.S. R&D, fueling the U.S. economy. Biopharmaceutical companies employ 800,000 Americans directly and support 4.7 million jobs nationwide.[46] In 2018 alone, the biopharmaceutical industry invested an estimated $102 billion in R&D,[47] more than any other industry.[48] In fact, the biopharmaceutical industry invests on average six times more in R&D as a percentage of sales than manufacturing industries overall.[49] IP is designed to, and does, foster both innovation and competition. IP protections and regulatory incentives give innovator companies a degree of certainty that their IP is protected—fostering innovation—while at the same time, the specifics of the invention covered by patents are published so others can learn from it and use it as the foundation for future invention and discovery—promoting competition. This public disclosure of inventions spreads knowledge and encourages others (i.e., competitors) to invent around existing patents and find new and different ways to solve problems and develop competing products.

The Nature of IP Protections for Biopharmaceutical Innovation

The benefits of IP incentives, including both patents and statutory exclusivity, with respect to innovation are significant in the biopharmaceutical industry. In the last decade alone, the FDA has approved nearly 900 new medicines, including the first medicine to treat the underlying cause of cystic fibrosis, the first vaccine to prevent cervical cancer, and the first ever gene therapies.[50] With sustained investments, our scientific understanding will continue to grow, creating new opportunities for profound advances against our most complex and costly diseases.

IP protections, including both patents and statutory exclusivity, are critical incentives for innovation, given the unique attributes of the biopharmaceutical R&D process:

- The R&D process involves a high level of scientific and regulatory uncertainty, with only 12 percent of investigational medicines that reach clinical trials ultimately receiving approval from the FDA.[51] Patent protection helps support continued future biopharmaceutical innovation over the long term, which includes providing the opportunity to earn revenue that can also compensate for the costly failures inherent in the biopharmaceutical R&D process.[52]

- Because research shows that R&D-intensive industries such as biopharmaceuticals are inherently riskier than non-R&D-intensive industries due to the uncertainty around R&D endeavors, investors require higher returns to compensate for those higher risks.[53] Benefits from R&D investments are uncertain and, if they occur at all, are realized over an extended time horizon, all of which increases the risk of such investments. Even standard measures of profitability show that the research-based biopharmaceutical industry's profits are in line with those of many other industrial sectors.[54]

- The significant time horizons and costs associated with biopharmaceutical R&D ranges from 10 to 15 years to develop a new medicine and an average of $2.6 billion dollars.[55] The growing cost of drug development is driven in part by increasing complexities in protocol design and manufacturing requirements, particularly for biologics. Today, clinical trials generate three times the data collected 10 years ago and clinical protocols have become significantly more complex, largely in response to regulatory expectations, contributing to growing R&D costs and challenges related to patient enrollment and retention.[56]

- Because certain initial patents are filed very early in the R&D process, at the time of FDA approval only half of the effective life of these patents may be left. Yet, FDA approval is not the end of innovation; rather, innovation continues throughout the biopharmaceutical life cycle.

IP protections for biopharmaceutical products are based on the concept of providing exclusive market access for a limited period as an incentive to support the substantial R&D efforts required for discovering and developing new and improved medicines. Patents confer the right to exclude competitors for a limited time within a given scope, as defined by patent claims. Once a new medicine's patent term and any statutory exclusivity protections have expired, generic equivalents, which require minimal capital investment, can enter the market. In the absence of effective IP protections, innovative biopharmaceutical companies would be unlikely to invest in developing innovative therapies.

IP Protections and Competition

IP protections do not impede competition in the U.S.; rather, they drive companies to innovate by providing a degree of assurance that companies may earn a return on an otherwise risky and costly investment in R&D. Moreover, IP protections do not block, but instead can foster, the entry of new competitors to market during the term of the patent. Similarly, patents or exclusivity that cover new formulations do not in any way extend the patents or exclusivity on previously approved formulations, or otherwise delay or block generic copies of the earlier formulations. Patents do not guarantee demand, nor do they prevent competition from nonidentical drugs that treat the same diseases and fall outside the protection of the patents. New medicines may enter the same therapeutic class with common mechanisms of action but different molecular structures (for example, different statins) or with differing mechanisms of action (such as calcium channel blockers and angiotensin receptor blockers). This means that drugs in the same class may have differences in effectiveness for patient subgroup populations, side effect profiles, and prices. The result is a market where products compete for patient and physician preferences as well as placement on drug formularies to the benefit of payers and patients. Since payers have strong tools to drive high generic use rates, new formulations will succeed in the marketplace only if they can demonstrate added value for patients.

It is important to keep in perspective that the term of a patent is 20 years from filing. Although a patent term adjustment (to compensate for patent office delays) or a patent term restoration (to compensate for drug approval process delays) may apply, the term of a patent cannot otherwise be extended.[57] Patents only provide a right to exclude as to the claimed invention. For example, once an initial compound patent expires, any competitor is free to practice that compound patent. Additional patent protection that might be obtained does not extend protection over that compound itself.

Patents Support Innovation Throughout the Biopharmaceutical Lifecycle

Patents touch nearly every facet of biopharmaceutical production and use, from the materials needed to produce a medicine, to the way it is made, to the active ingredient or component that produces its biological effect, to formulations of it, to new uses of it; the result of this breadth of innovation is that most medicines are associated with many patents.

The types of patents covering biopharmaceuticals include:

- Patents covering the active ingredient or component (the part of the medicine that produces its biological effect).

- Drug product patents, which refer to the particular form in which the medicine is delivered to a patient. New dosage forms for already FDA-approved medicines can increase patient adherence to therapy, ensure a proper dose is taken, and improve quality of life for patients who must use the medication on a prolonged basis. In turn, these innovations may result in improved health outcomes and a reduction in unnecessary hospitalizations. As an example, an injectable treatment for schizophrenia has allowed for less frequent dosing than previous forms with the potential to increase patient compliance. The long-acting form allows the medicine to remain within a therapeutic range for an extended period, helping patients better manage their disease symptoms.

- Methods of use/treatment patents. Knowledge and understanding of a medicine continue to build over time, through additional study and collection of data. This additional research can culminate in approval of new uses of medicines in different patient populations, conditions, and disease states, expanding treatment options for patients. As an example, medicines initially developed for use in rheumatoid arthritis have been shown to also help treat other autoimmune conditions that share similar molecular pathways, including Crohn's disease and ulcerative colitis. In oncology, for example, research is often under way on multiple additional indications at the time of approval of the initial indication, with post-approval clinical research often demonstrating significant clinical benefit of the therapy in a different disease, stage of disease or population.

- Methods of manufacturing patents, which cover innovations in the process or steps to manufacture increasingly more complex medicines. Advances in manufacturing processes can improve the safety and effectiveness of medicines, such as by removing potential impurities that could present safety issues (e.g., carcinogenicity, genotoxicity, immunogenicity). These innovations similarly require R&D incentivized by IP protections. In some cases, innovator firms (and, for that matter, biosimilar firms) may have developed more precise analytical methods, as well as more precise understandings about the effects of different manufacturing method changes. For R&D intensive industries, the manufacturing process is a key factor in developing new products. That's because in these industries, product and process innovation are often intertwined. Manufacturers justifiably may seek to protect these innovations, while also disclosing these processes to the public, through patents. Although biosimilar competitors may need to consider how they will proceed in light of the patents, one approach is inventing around the methods disclosed in the patent. As noted previously, prospective applicants can also choose to challenge the patents or their applicability through the process articulated in the BPCIA.

  In contrast to patents that cover the composition of a new compound, new uses and new methods of manufacturing can be invented at any point in the product lifecycle, and thus patent applications for them can also be filed throughout the product lifecycle. For instance, new methods of manufacture that reduce the potential for immunogenicity are often invented years after a biologic is discovered or has obtained regulatory approval. In addition, manufacturers may invent novel methods for purifying proteins that are more efficient or allow for more precise recovery of specific proteins. Such advances in manufacturing methods should be incentivized to maximize product quality, safety, and effectiveness and ensure efficient delivery of a consistently safe and effective product to patients.

Potential Barriers to Competition

PhRMA strongly supports policies that foster a robust, competitive market for generic and biosimilar medicines while providing needed incentives for continued biopharmaceutical innovation. Robust, competitive markets for generic drugs and biosimilars will play an increasingly important role in supporting affordable care. The natural evolution of medicines is that, after an innovator undertakes the time-consuming, uncertain, and expensive development process and obtains FDA approval, it enjoys an appropriate period of IP protections, including both data protection and patent protections, following which generic or biosimilar versions, as appropriate, can be approved. Indeed, this is the very cycle that Hatch-Waxman and BPCIA were intended to encourage. The introduction of innovative therapies provides patients with new treatment options and leads to competition where there are multiple alternatives in a given therapeutic class.

There are several areas where competition could be enhanced without reducing incentives for innovation, including:

- Advancing meaningful rebate reform
- Addressing certain types of patent settlements
- A balanced approach to address "product hopping"

- Continuing to foster timely generic and biosimilar approval and market entry.

Advance Meaningful Rebate Reform

As a result of rebates and discounts that pharmaceutical companies pay to health insurance companies, middlemen like pharmacy benefit managers (PBMs), the government, and others, net prices for brand medicines are, on average, 44 percent lower than the list price.[58] However, these rebates and discounts often do not reach Medicare or commercially insured patients at the pharmacy counter, particularly for patients with high deductibles and coinsurance. If insurance companies and middlemen do not pay the full price for medicines, patients should not have to either. These rebates and discounts should be shared with patients at the pharmacy counter to ensure that products compete on price and patients benefit from that competition.

Address Certain Types of Patent Settlements

Congress enacted as part of the Hatch-Waxman Act a complex framework governing the timing of generic applications that respects IP and specifically contemplates patent litigation. Under this framework, innovator companies submit patent information to FDA for publication, or "listing," in FDA's Orange Book. A generic applicant needs to certify with respect to listed patents whether it seeks to market its proposed generic product prior to expiration of the patent or after expiration. If it seeks to market its product prior to patent expiration, it generally must file a "Paragraph IV certification" with FDA in which it certifies its belief that the patent is invalid or would not be infringed by the generic product, and it must notify the innovator company of that certification. The innovator company can then bring suit under a special cause of action for patent infringement that allows for litigation prior to the generic marketing its product. If the suit is brought within 45 days of the innovator receiving notice of the Paragraph IV certification, FDA cannot approve the generic application for 30 months (or sooner if the generic is successful in the litigation) so that the court can address the patent disputes prior to marketing of the generic product (although FDA continues to review the generic application).[59] Hatch-Waxman also provided an incentive to generics to challenge patents under the Hatch-Waxman process in the form of 180-day generic exclusivity.

In general, if the generic applicant wins in litigation, FDA can approve the generic product; but if the innovator wins, FDA cannot approve the generic product for marketing until patent expiration. There can be many generic challengers for individual products, so Hatch-Waxman can lead to a substantial amount of litigation. Like other patent infringement litigation, the parties may choose to settle the case, with such settlements generally leading to generic companies entering the market prior to patent expiration, and potentially prior to when they could have entered if the litigation had continued. Settling such litigation is not surprising given the burden of litigation and the uncertainty for both innovators and generics.

The FTC and some other stakeholders have asserted that there are anticompetitive settlements in which innovator companies have provided cash payments and generic companies have delayed marketing their products. Under the 2013 Supreme Court decision in *FTC v. Actavis*, the FTC can seek to enforce the existing law against patent settlements with cash payments under the rule of reason – a fact-based inquiry. The FTC has asserted a broader view, and there is legislation pending that would create a presumption that certain agreements are anticompetitive, including agreements entered since 2013. There have also been bills introduced in several states, and California has passed restrictive legislation.

PhRMA supports addressing patent settlements with federal legislation to ensure generic, biosimilar and innovator companies can resolve patent litigation and allow generic and biosimilar medicines to enter the market prior to expiration of innovators' patents, without applying new policies retroactively to previous agreements or restricting companies' ability to enter into pro-competitive agreements in the future. We are committed to working with the Committee to address concerns in this area and promote competition.

A Balanced Approach to Addressing Product Hopping

There have been situations in which companies have been held liable after taking steps in conjunction with the introduction of new versions of products that were found to be anticompetitive. Legislation is pending that would create a presumption of anticompetitive effect in situations defined as "hard switches" or "soft switches." A "soft switch" as defined in the legislation, for instance, can include situations in which a company develops a new product and takes actions that may "unfairly disadvantage" the earlier version of the product, even though that earlier version is still marketed. The legislation, however, gives little guidance on what activities could constitute "unfairly disadvantaging" the earlier product. This could put a cloud over many types of innovations after an original FDA approval that render a medicine safer or more effective, or improve patient care or quality of life. If Congress acts, it should do so in a balanced way that supports continued improvement to medicines that bring new benefits for patients, while addressing potential anticompetitive behavior.

Continuing to Foster Timely Generic and Biosimilar Approval and Market Entry

PhRMA continues to support a robust competitive marketplace that includes the timely approval and entry of generic and biosimilar medicines. The FDA has taken meaningful steps to streamline and expedite the generic drug approval process, such as promoting more high-quality generic applications. The agency, for example, publishes a list of off-patent, off-exclusivity drugs without approved generics and has updated its internal procedures to allow expedited review for some generic drugs. The FDA has also enacted numerous guidance documents to provide clarity to biosimilar manufacturers regarding the data needed to support applications to FDA for biosimilar and interchangeable biologic products.

Market Distortions in the Distribution and Payment System for Prescription Medicines

In this section, we briefly discuss three areas of market distortions that negatively impact patients which warrant further examination: misaligned incentives in the distribution and payment system for prescription medicines, distortions related to the 340B program and consolidation in hospitals, and increased shifting of costs onto patients.

Misaligned Incentives in the Part D Rebate System

The Part D program has been a resounding success since its start in 2006. According to Congressional Budget Office (CBO) estimates, total Part D costs are 45 percent ($349 billion) lower than projected for the initial 2004 to 2013 forecast period.[60] The Medicare actuaries report that the Part D base beneficiary premium in 2021 is less than $1.00 more than it was in 2006, the first year of the program's operation.[61]

Powerful Part D purchasers already negotiate discounts and rebates with manufacturers. The Medicare Trustees report that "many brand-name prescription drugs carry substantial rebates,"[62] which have increased each year of the program.[63] These negotiations, on average, yield larger rebates for brand medicines in Part D than are seen in the commercial insurance market. Specifically, the Altarum Institute found that Part D plans negotiate larger rebates on brand medicines within Part D (31 percent) as compared to commercial insurance (16 percent).[64] CBO says Part D rebates and discounts are "somewhat larger than the average rebates observed in commercial health plans."[65] And the Government Accountability Office (GAO) found that Part D plans had negotiated rebates and discounts for 99 percent of the brand medicines most commonly used by Part D beneficiaries. Further, for the top 200 brand medicines ranked by total Part D spending, GAO found that rebates and discounts nearly doubled from 2014 to 2016.[66]

Although these privately negotiated rebates can result in substantial savings, Part D plans still require most beneficiaries to pay cost-sharing based on the full price of their medicine, even in cases where the plan receives a substantial rebate from manufacturers. In fact, a recent analysis shows that 92 percent of Part D beneficiaries' out-of-pocket spending is based on the list price rather than the discounted price their plans get.[67]

To improve patient affordability, insurers and PBMs should share more of the discounts and rebates they negotiate with biopharmaceutical companies directly with patients at the point of sale. Once medicines are researched, developed, and approved for use, the process by which prescription medicines move from biopharmaceutical manufacturers to patients involves multiple stakeholders and numerous financial transactions. This process has evolved significantly in recent years, as supply chain entities have grown to play a larger role in drug distribution and payment. Three large, sophisticated PBMs manage 77 percent of all prescriptions filled.[68] They use brand competition to obtain discounts from manufacturers and take full advantage of the presence of generics to drive savings. In fact, the use of generic medicines, which account for 91 percent of prescription medicines dispensed in the United States, saved nearly $2.2 trillion between 2010 and 2019,[69] and these dynamics will continue to produce savings.

Competition from generics and biosimilars will result in an estimated $128 billion reduction in U.S. brand sales through 2025.[70] Additionally, biosimilar competition in the biologics market will increase substantially over time as the market matures.[71] Between 2016 and 2020, loss of exclusivity (LOE) lowered brand spending by $84 billion and LOE is expected to decrease brand spending by $128 billion through 2025, with $39 billion coming from branded biologic LOE.[72] There is no similar type of cost containment for other health care services.

Consolidation and increased negotiating power give middlemen like PBMs leverage to extract growing price concessions from manufacturers. The magnitude of these rebates, discounts, and other reductions in price have more than doubled since 2012, totaling over $187 billion in 2020.[73] For certain medicines used to treat chronic conditions like asthma, high cholesterol, hepatitis C, and diabetes, these discounts and rebates can reduce list prices by more than 80 percent.[74] According to a study by the Berkeley Research Group, on average, nearly half of all spending on brand prescription medicines in 2018 went to someone other than the biopharmaceutical manufacturers who researched, developed, and manufactured the medicines.[75]

Even though payers often receive deep discounts on a brand medicine's price, they rarely directly pass along those savings to the patients obtaining those medicines at the pharmacy counter. Instead, health plans typically use some portion of negotiated rebates to reduce monthly premiums for all enrollees. As the actuarial firm Milliman has pointed out, this dynamic results in a system of "reverse insurance" where payers require sicker patients using brand medicines with rebates to pay more out of pocket, while rebate savings are spread out among all health plan enrollees in the form of lower premiums.[76] Asking sicker patients with high medicine costs to subsidize lower premiums for healthier enrollees is the opposite of how health insurance is supposed to work.

This problem is particularly striking for patients with diabetes taking insulin. Robust competition among insulin manufacturers has resulted in increasing levels of discounts and rebates that have resulted in declining net prices over the past several years.[77] That is because payers leverage competition among a broad range of long-, short-, rapid-acting insulin to negotiate lower prices. These dynamics lower the list price of brand diabetes medicines by 83 percent on average.[78] Although media reports commonly give the false impression that biopharmaceutical companies retain all revenue from list price increases, flat net price growth indicates that all or almost all of insulin list price increases are returned to payers, the government, and other medicine supply chain entities through rebates, fees, or other discounts.

While robust competition in the market has been successful in constraining net prices for insulins, medicine supply chain intermediaries have incentives to favor high list prices and large rebates, leading to affordability challenges for patients who pay cost sharing based on the list price. Helping patients access the treatments they need by passing through rebates at the point of sale to lower patient cost sharing could improve medicine adherence for conditions like diabetes, which could ultimately generate savings by reducing costly avoidable health complications. A recent study by IHS Markit found that passing through a share of rebates to Medicare Part D patients taking diabetes medicines could reduce overall health care spending (including spending in Parts A and B) for Medicare beneficiaries with diabetes by $20 billion over the next 10 years.[79]

A final rule from the U.S. Department of Health and Human Services' (HHS) Office of the Inspector General (OIG)[80] is an important step towards an improved Part D program, by replacing protections for drug rebates with protections for passing along those discounts directly to patients. OIG reports that, on average, Medicare Part D beneficiaries who do not receive low-income subsidies (LIS) (non-LIS) would pay 10 to 19 percent *less* in cost sharing over the next 10 years under the revisions that would encourage upfront discounts.[81] And patients who take brand medicines with relatively large rebates, such as medicines for rheumatoid arthritis , would be likely to see larger-than-average reductions in out-of-pocket costs because they would now directly benefit from those negotiated rebates.[82]

The principles underlying the rebate rule reforms could restore incentives to favor lower net cost medicines while strengthening incentives to negotiate deep discounts on medicines. Part D plans will have strong incentives to minimize costs in the absence of retained rebates. As Milliman notes, plans would be incentivized to achieve lower net costs to minimize premium increases and maintain LIS auto-enrollment.[83] Actuaries have also suggested that under the changes finalized by the OIG, some manufacturers "may have more success marketing biosimilars in Part D if manufacturer rebates are eliminated," due to the incentives for plans to achieve lower net costs.[84]

Distortions and Lack of Competition in the Provider Market

Hospitals are a large contributor to health care costs. In 2019, hospital care is projected to total $1.25 trillion and represent nearly a third of health care spending; this number is expected to increase to nearly $2 trillion by 2027.[85] As such, an important factor in rising health care costs is increasing consolidation and mark-ups in the cost of care delivered in the provider market. Hospitals, for example, substantially mark up new medicines. Nearly one in five hospitals marks up medicine prices 700 percent or more.[86] This means that if a hospital purchased a medicine for $150, a 700 percent markup could result in patients being billed $1,050 for that medicine. Additionally, the analysis[87] found that 320 hospitals – eight percent of those included in the study – marked up some medicine prices by 1,000 percent or more.

Hospital consolidation and the resulting mark-ups increase health care costs for patients and providers, but there is no evidence that this consolidation improves the quality of care.[88] The Medicare Payment Advisory Commission (MedPAC) recently concluded that the "preponderance of evidence suggests that hospital consolidation leads to higher prices."[89] The 340B program is one factor leading to more vertical provider consolidation due to the incentives 340B creates for hospitals to shift care from community-based physicians to higher-cost settings.[90] Congress created the 340B program to provide discounts on outpatient drugs dispensed by federal grantees and hospitals that serve large numbers of uninsured or otherwise vulnerable patients. To achieve that goal, hospitals and safety net clinics that meet certain eligibility criteria are entitled to steep discounts for medicines for eligible 340B "patients." Disproportionate share hospitals (DSH) have come to dominate sales in the 340B program and recent data shows that hospitals now account for about 80 percent of 340B sales.[91] Current program rules are outdated and overly broad, oftentimes allowing hospitals to make a large profit by dispensing 340B medicines that were obtained at the steeply discounted 340B ceiling price and charging patients the full list price. By doing so these hospitals fail to pass along any of the 340B discount to lower a patient's drug costs or use their profits to help increase hospital charity care.

Hospitals have also leveraged their ability to generate revenue from 340B discounts to purchase physician groups.[92] Once these practices are purchased, hospitals oftentimes register these outpatient clinics as 340B discounts as child sites, even if though those sites do not lower drug costs or provide any free or discounted care to a 340B patient. One economist recently testified before this same committee and stated that "it can therefore be very profitable to hospitals and physician practices for hospitals to own physician practices that provide a lot of physician-administered drugs to their patients, since the hospital obtains the drugs at a substantial discount through the 340B program, and both the discount and the revenues are captured by the combined hospital-physician practice."[93] These shifts in ownership and the site of treatment not only undermine community-based physician practices but also drive concentration in provider markets, leading to higher prices for payers, the government, and patients.[94]

Hospitals' rapid acquisition of physician practices also enables them to demand high prices from commercial payers, driving up spending for all services. From 2004 to 2011, hospital ownership of physician practices doubled from 24 percent to 49 percent.[95] As a result, insurers pay higher prices for equivalent services that previously were delivered in less-expensive independent physician offices.[96] Additionally, expansion of 340B to for-profit contract pharmacies is driving increased utilization through vertically integrated systems of pharmacies, PBMs and health plans. A report documenting this also notes that the large profits for 340B contract pharmacies may be creating additional incentives for vertical consolidation across these different types of entities and shifting 340B profits to large, for-profit corporations.[97] A range of changes are needed to strengthen the 340B program and ensure it no longer serves as a profit-center that fuels further health care consolidation.[98]

Increased Cost-Shifting to Patients

A growing distortion in the market is the increased shifting of costs to patients. Patients pay cost sharing for health care items and services, including prescription medicines, through deductibles, copays, and coinsurance. When a patient fills a prescription in the deductible phase, the patient pays the entire list price of the medicine up to the deductible amount determined by the health plan. Patients with copays pay a fixed amount for each prescription (e.g., $30), while those with coinsurance typically pay a percentage of the medication's total list price (e.g., 30 percent).

In the last decade, in the commercial market, the share of patient out-of-pocket drug spending represented by coinsurance has more than doubled, while the share accounted for by deductibles has tripled.[99] Since 2006, deductibles for patients in employer-sponsored health plans have increased by 300 percent.[100] Between 2007 and 2017, patient out-of-pocket spending on coinsurance has increased 74 percent, while spending on copays has decreased.[101] The share of employer-sponsored health plans requiring a deductible for prescription medicines has more than doubled from 23 percent in 2012 to 52 percent in 2017.[102] As one recent analysis shows, patients are required to pay 11 percent of overall pharmaceutical costs versus only 2 percent of hospital inpatient costs – even though medicine can help keep patients out of the hospital.[103]

Deductibles and coinsurance can leave patients with high and often unpredictable costs, particularly for their medicines. Average commercially insured patient out-of-pocket costs for deductible and coinsurance claims for brand medicines are much higher than copay claims.[104] In 2019, half of commercially insured patients' out-of-pocket spending for brand medicines was for medicines filled while a patient was in the deductible or with coinsurance.[105] Patients with chronic conditions are disproportionately impacted by high out-of-pocket costs.[106]

In Medicare Part D, there has been a substantial increase in the use of coinsurance and complex, multi-tiered formularies. Today, 86 percent of stand-alone Part D plans (PDPs) use formularies with five coverage tiers, while 14 percent are now using a sixth tier.[107] The percentage of PDPs with 3 coinsurance tiers is increasing substantially, from 11 percent in 2020 to 23 percent in 2021. As a result of this shift, 62 percent of all medicines covered by PDPs are now on a coinsurance tier.[108]

When patients receive medical care from an in-network hospital or physician, deductible and coinsurance payments are based upon discounted rates negotiated between the health plan and the provider. Yet this is not the case for prescription medicines. Health plans (and the PBMs that represent them) negotiate discounts on brand medicines, but the discounts are given in the form of rebates paid directly to the health plan or PBM after the medicine is purchased by the patient. These discounted prices are typically not the prices used to calculate patients' deductibles or coinsurance amounts at the time they fill their prescriptions; instead, their cost sharing is generally calculated by the health plan based on the medicine's full list price.

Research shows that rebates paid by biopharmaceutical companies often substantially reduce the cost of brand medicines.[109] However, since list prices do not reflect rebates, these savings may not be directly passed on to

patients through lower cost sharing, and patients' out-of-pocket costs for prescriptions filled within the deductible or with coinsurance may be higher than they otherwise would be if they were based on the discounted cost of the medicine. Thus, the growing use of deductibles and coinsurance for medicines has exposed patients to out-of-pocket costs based on undiscounted list prices, creating affordability challenges for many, including the most vulnerable.[110]

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As the Committee considers policy solutions, we urge the Committee to avoid broad policies that would chill innovation, destabilize important incentives for development of new medicines, and negatively impact patient access to innovative therapies and cures. We hope the committee recognizes how IP protections enabled biopharmaceutical research companies to move quickly and effectively against COVID-19 and are critical to our ongoing efforts to increase manufacturing capacity and find new ways to fight the virus. Instead of focusing on proposals that undermine the competitive marketplace for medicines and incentives for innovation, we encourage a focus on addressing market distortions and providing pragmatic solutions, including modernizing the drug discovery and development process and removing barriers that limit paying for value. PhRMA appreciates the opportunity to testify and looks forward to continuing to engage with the Committee on these critically important issues.

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