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Chairman Graham, Ranking Member Feinstein, and Members of the Committee, thank you for inviting me to participate in today's hearing. Understanding the role of intellectual property (IP) in fostering innovation and competition in the biopharmaceutical marketplace is a critical component of the discussion about improving patient access and affordability and I appreciate the opportunity to explore this topic with you in depth.

The Pharmaceutical Research and Manufacturers of America (PhRMA) represents the country's leading innovative biopharmaceutical research companies, which are devoted to discovering and developing medicines that enable patients to live longer, healthier, and more productive lives. The biopharmaceutical sector is one of the most research-intensive industries in the U.S.: since 2000, PhRMA member companies have invested more than half a trillion dollars in the search for new treatments and cures, and R&D investments totaled almost \$90 billion by industry in the U.S. alone in 2016.

PhRMA appreciates the Committee's leadership in exploring opportunities to enhance competition in the health care 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 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 distortion in the health care marketplace.

Medicines are Transforming the Trajectory of Disease

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 nearly 60 percent.¹ 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.²
- *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 an 88 percent decline in death rates in the United States.³
- *Hepatitis C*: More recently, we've seen a remarkable transformation in treatment of another viral disease: hepatitis C. Just six years ago, the only available treatment cured just half of patients and caused debilitating side effects. Today, a broad range of treatments with minimal side effects and cure rates approaching 100

percent are available for patients with all forms of the disease.^{4,5} Looking forward, researchers project that with improved screening and today's cures, hepatitis C will be a rare disease by 2036.⁶

- *Cancer*: New medicines are also a driving force behind gains in the life expectancy of cancer patients. Since peaking in the early 1990s, cancer death rates in the United States have declined 26 percent.⁷ Researchers attribute 73 percent of these gains to new treatments, including new medicines.⁸ 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.⁹

Researchers are pursuing cutting-edge research and novel scientific strategies to continue to drive therapeutic advances for patients. There are currently about 7,000 medicines in clinical development globally with the potential to impact U.S. patients.¹⁰ And across the medicines in the pipeline, 74 percent have the potential to be first-in-class treatments.¹¹ Medicines in development include:¹²

- *Neurological disorders*: These disorders affect a broad range of conditions affecting the brain and nervous system—for example, epilepsy, migraine headaches, multiple sclerosis, Parkinson's disease, and Alzheimer's disease. There are more than 500 medicines in development for neurological disorders. One exciting cell therapy approach for amyotrophic lateral sclerosis (ALS) involves extracting stem cells from patient bone marrow and customizing the cells to help support the survival of neurons once the cells are returned to the patient.¹³
- *Cancer*: In addition to the adaptive cell therapy and gene therapy approaches that are just beginning to transform the lives of patients, several novel approaches – including antibody-drug conjugates, immune checkpoint modulators, metabolic immunotherapies, and vaccines – are showing tremendous promise in the pipeline against a broad range of cancers. Today, there are 1,120 medicines and vaccines currently in development for cancer.¹⁴
- *Heart disease and stroke*: Cardiovascular disease is the leading cause of death in the United States, affecting 92.1 million American adults. There are currently 200 medicines in development for heart disease and stroke. One promising investigational medicine is a non-viral gene therapy that targets a tissue and regeneration pathway that promotes cardiac function, cell survival, and the repair of injured heart tissue in patients with ischemic heart failure.¹⁵

Today's biopharmaceutical pipeline has tremendous promise and represents a new frontier of research with the potential 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 recently by the 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."¹⁶ 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 addition, Congress, recognizing the need to provide approval pathways that foster competition through the market entry of generic and biosimilar medicines while also maintaining incentives for innovation, has enacted two statutory frameworks that simultaneously reward innovation and establish 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.

The Hatch-Waxman Act was intended both to increase access to generic medicines and to preserve incentives for innovation. As such, there is a balance between innovation and access guiding implementation of it. The Hatch-Waxman Act 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 did not have an abbreviated pathway for approval of generic copies of drugs approved after the 1962 amendments to the Federal Food, Drug, and Cosmetic Act after 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,¹⁷ overturning a Federal Circuit decision to the contrary,¹⁸ and seek FDA approval to market products immediately upon patent expiration, or even prior to patent expiration if they 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 attempt 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 face generic competition at between 12 and 13 years after brand launch even though the basic patent term is 20 years.¹⁹ Over the 34 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 as soon as possible under the statute—in the case of a new chemical entity, as early as 4 years after FDA approval.²⁰

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.
- 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 in a new class of hepatitis C treatments multiple competitors entered the market, resulting in lower prices and improved clinical effectiveness. The competition was so fierce that Express Scripts, the U.S.' largest PBM, now touts that hepatitis C treatment is less expensive here than in other western countries thanks to their aggressive negotiation.²¹ In the case of PCSK9 Inhibitors, according to public reports, large purchasers have used their market power to negotiate publicly reported discounts on these biologic medicines ranging as high as 50 percent. And today, list prices for the class have dropped by approximately 60 percent.
- The competitive market is structured to take maximum advantage of savings from brand competition. Brand medicines competition 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 a medicine was alone in its class was 2.3 years.²²

- 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 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.²³ 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 \$105 billion from 2018 to 2022.²⁴

The BPCIA was enacted in 2010 and was intended to strike a balance between providing access to biosimilar medicines and preserving incentives for innovation. 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 created by the Hatch-Waxman Act and BPCIA litigation procedures differ, they both allow for, and naturally lead to, premarket patent litigation.

While the BPCIA is less than a decade old, and biosimilar development is significantly more complex and expensive than generic drug development, the benefits of the BPCIA on innovation and competition are already being seen. The FDA approved the first biosimilar product for marketing in the U.S. in March 2015 and, as of April 2019, 19 biosimilars have been approved in the U.S. Recognizing that the European Union (EU) has had a biosimilars pathway in place for a longer 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 and include costs and complexities related to manufacturing and scale up of manufacturing, lack of awareness and education among prescribers and payers of biosimilars, and the lack of final FDA guidance around interchangeability. Final interchangeability guidance is particularly important as it will provide clarity to biosimilar manufacturers regarding demonstrating that a proposed therapeutic protein product (proposed interchangeable product or proposed product) is interchangeable with a reference product for the purposes of submitting a marketing application or supplement. A determination of interchangeability by FDA would mean that in most states the biological product may be substituted for the reference product without the intervention of the health care provider who prescribed the reference product.

FDA is implementing an action plan aimed at accelerating the market entry and uptake of these extremely complex products that are in many ways more difficult to develop and produce compared to 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 many aspects of the FDA's Biosimilars Action Plan, particularly FDA's continued efforts to develop "effective communications to improve understanding of biosimilars among patients, clinicians, and payors."²⁵ These efforts will raise 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. 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. Recent studies project that biosimilars could reduce spending on biologics by \$25 billion to \$150 billion over the next 10 years.²⁶

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 increasing 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.

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 numerous 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.²⁷

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.²⁸ 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 just 14 percent of total U.S. health care spending.²⁹ Biopharmaceutical innovator companies, which develop new medicines that save and improve patients' lives, accounted for less than one-half of all spending on prescription medicines—or about 7 percent of total health care spending in 2015.³⁰

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 medicines results in sizable discounts in the prices of brand medicines and shifts utilization from brand medicines to generics and biosimilars.³¹ In 2018, prices for brand-name medicines increased just 1.5 percent after discounts and rebates, less than the rate of inflation.³² This trend of low growth is expected to continue; between 2019 and 2023, IQVIA projects annual net price growth for brand-name drugs will be just 0 to 3 percent.³³

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 7,000 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.³⁴ The U.S. develops more new medicines than the rest of the world combined,³⁵ 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.³⁶ In 2016 alone, the biopharmaceutical industry invested an estimated \$90 billion in R&D, more than any other industry.³⁷ In fact, the biopharmaceutical industry invests on average six times more in R&D as a percentage of sales than all other manufacturing industries.³⁸ IP protections and competition are by and large a product of the market economy. 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 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 more than 500 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.³⁹ With sustained investments, our scientific understanding will continue to grow, creating new opportunities for profound advances against our most complex and costly diseases. As just one example, the discovery of a medicine that could delay the age of onset of Alzheimer's disease by five years would mean 1.6 million fewer Americans would have Alzheimer's, in turn saving \$100 billion in annual medical costs by 2030.⁴⁰

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.⁴¹ Patent

protection helps support continued future biopharmaceutical innovation over the long term, including by providing the opportunity to earn revenue that can also compensate for the costly failures inherent in the biopharmaceutical R&D process.⁴²

- 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.⁴³ 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.⁴⁴
- 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. The growing cost of drug development is driven in part due to increases in protocol requirements as well as manufacturing complexities, particularly for biologics. Protocol design for clinical trials has increased in complexity, which has contributed to growing R&D costs and challenges related to patient enrollment and retention.⁴⁵
- 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 but rather innovation continues throughout the biopharmaceutical life cycle.

IP protections 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 regulatory exclusivity provisions have expired, generic equivalents, which require minimal capital investments, can enter the market. In the absence of 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). 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. Medicines in the same class compete through quality and price for preferred placement on drug formularies and physicians' choices for patient treatment.

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.⁴⁶ 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 Supporting 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. An overview of the various aspects of a medicine that may be covered by patents is provided below:

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 refers 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 continues 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 finding in many instances that a therapy demonstrates significant clinical benefit in a different disease or different stage of disease.
- Methods of manufacturing patents, which cover innovations in the process or steps to manufacture these increasingly more complex medicines. Advances in manufacturing processes can improve the safety and effectiveness of medicines, for example 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, originator 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 most of these industries product and process innovation are 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 BPCIA. Unlike for 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. This reflects the numerous innovations that have occurred to study the biologic as a treatment for additional diseases and to streamline and improve manufacturing processes. New methods of manufacture that reduce the potential for immunogenicity are often invented years after the biologic was discovered, for example, in the process of scaling up to commercial manufacture or even based on continuing improvements that occur after a biologic has been submitted for or 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. Advances in such 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, which are described below:

- Ensuring access to product samples
- Addressing certain types of patent settlements
- Increasing competition for older, off-patent medicines

Ensure Access to Product Samples

PhRMA appreciates the balance struck by the Hatch-Waxman Amendments under which generics are approvable under an abbreviated pathway after a period of IP protection.⁴⁷ Although it is a different framework, the BPCIA relies on a similar premise allowing for the approval of biosimilars once reference product exclusivity has lapsed.⁴⁸ These regimes operate from the starting proposition that IP rights are key to innovation and thus must be respected, but once applicable protections have expired, generics and biosimilars should be eligible for approval. To ensure this is possible, reference product samples should be reasonably available under terms consistent with patient safety for the bioequivalence and biosimilarity testing required for their approval and licensure, when permitted under statute.

Reference product sponsors should not withhold samples to delay generic or biosimilar entry. We support the overall intent of the Creating and Restoring Equal Access to Equivalent Samples (CREATES) Act but hope to see changes that preserve the role of FDA in access decisions for products with Risk Evaluation and Mitigation Strategy (REMS) with Elements to Assure Safe Use (ETASU) and ensure the bill achieves its objectives without incentivizing frivolous litigation. Specifically, we support language that codifies within the FDCA an authorization process for access to samples for products with REMS with ETASU. We also would encourage safeguards in any new cause of action including affirmative defenses for license holders that offer samples at commercially reasonable terms as well as statutory assurance that providing samples would not violate REMS.

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 the process, innovator companies submit patent information to FDA (known as “listing”) for publication 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 issues prior to marketing of the generic product. Hatch-Waxman also provided an incentive to generics to challenge patents under the Hatch-Waxman process in the form of 180-day generic exclusivity.

The litigation proceeds in U.S. district court. In general, if the generic 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.

PhRMA supports the need to promote generic competition after the expiration of relevant patents and exclusivities. We are committed to working with the Committee to address concerns and promote competition. PhRMA has concerns, however, about legislation that would change the law and apply retroactively to previously entered into agreements and that would create presumptions and unfairly restrict the ability of companies to demonstrate that agreements are procompetitive.

Increase Competition for Older, Off-Patent Medicines

Over the past few years, rapidly rising prices for some older medicines whose patents and statutory exclusivity protections have expired have garnered the attention of policymakers, media and patients. In multiple instances, decades old medicines suddenly increased in price. Generally, the price of a medicine drops when IP protection ends, and a generic copy enters the market. However, in recent instances the opposite has occurred: prices for older, off-patent medicines have increased, rather than decreased. This has occurred even when there were no regulatory or patent barriers prohibiting entry of a competitor. For example, in the well-publicized case of Turing Pharmaceuticals' drug Daraprim, a small population size for the product which had been on the market for decades with no IP protections enabled Turing to dramatically increase the price of the product, knowing there were insufficient incentives for generic to enter the market given the small population size and costs to enter the market.

Through the enactment and implementation of the FDA Reauthorization Act of 2017 (FDARA), both Congress and FDA have taken several important steps to address these unique situations and increase competition for older, off-patent medicines. FDARA established additional incentives, in the form of 180-day exclusivity, for certain "competitive generic therapies." Applications eligible for this 180-day exclusivity are those drugs designated as competitive generic therapies based on only one drug being listed in active section of Orange Book *and* where that drug has no remaining patent or exclusivity protection. The exclusivity runs against subsequent generic products to encourage generic manufacturers to enter the market in the first place. As of February 2019, FDA had granted more than 100 competitive generic therapy designations, noting that continued successful implementation of the competitive generic therapy designation process is a "key step . . . to ensur[ing] there's adequate competition in the market place."⁴⁹

While Congress and the FDA have taken steps to facilitate greater competition where no IP protection remains for the brand product and there is inadequate generic competition, additional incentives may be

needed to inject greater competition and lower costs into the market in these circumstances, including but not limited to expediting the review of generic drug applications and inspection of the generic manufacturing facilities; waiving existing application fees; and establishing a list on FDA's website of contract manufacturers who could provide manufacturing capacity if needed. Through such reforms, generic manufacturers would have additional incentives and predictability to enter markets where there is currently inadequate generic competition and no remaining IP protection on the brand product. In turn, this would allow for greater generic competition in the marketplace. Research consistently demonstrates that increased competition increases patient access to affordable medicines and reduces overall health care spending.

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

Since the start of the Part D program in 2006, it has been a resounding success. 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.⁵⁰ Average monthly beneficiary premiums are estimated to be about \$32.50 in 2019,⁵¹ substantially lower than the \$54.47 originally projected.⁵² Powerful Part D purchasers already negotiate discounts and rebates with manufacturers. The Medicare Trustees report that "many brand-name prescription drugs carry substantial rebates,"⁵³ and have increased each year of the program.⁵⁴ However, patients do not always benefit from these rebates, resulting in affordability challenges for some Part D beneficiaries taking brand-name medicines with large manufacturer discounts.

To improve patient affordability, insurers and pharmacy benefit managers (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 over 75 percent of all prescriptions filled.⁵⁵ 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 accounts for 90 percent of prescription medicines dispensed in the U.S., saved \$1.79 trillion between 2008 and 2017,⁵⁶ and these dynamics will continue to produce savings. Between 2019 and 2023, competition from generics and biosimilars will result in an estimated \$95 billion reduction in U.S. brand sales.⁵⁷ Additionally, biosimilar competition in the biologics market will increase substantially over time as the market matures.⁵⁸ 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 \$166 billion in 2018.⁵⁹ For certain medicines used to treat chronic conditions like asthma, high cholesterol, hepatitis C, and diabetes, these discounts and rebates can reduce list prices by as much as 30 to 70 percent.⁶⁰ According to a study by the Berkeley Research Group, on average, more than one-third of the initial list price of a medicine is rebated back to insurance companies, PBMs, and the government, or retained by other stakeholders along the biopharmaceutical supply chain.⁶¹

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 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.⁶² Asking sicker patients with high medicine costs to subsidize 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 kept net prices flat to declining over the past several years.⁶³ That is because payers leverage competition among a broad range of long-, short-, rapid-acting insulin to negotiate lower prices. These dynamics can lower the net price of insulin by 70 percent or more.^{64, 65} 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.⁶⁶

A proposed rule from the U.S. Department of Health and Human Services' (HHS) Office of the Inspector General (OIG)⁶⁷ is an important step towards an improved Part D program. 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 Administration's proposed system to encourage upfront discounts.⁶⁸ And patients who take brand medicines with relatively large rebates, such as medicines for diabetes, would be likely to see larger-than-average reductions in out-of-pocket costs because they would now directly benefit from those rebates.⁶⁹

The principles underlying the rebate rule could restore incentives to favor lower 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.⁷⁰ Actuaries have also suggested that under the changes proposed 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.⁷¹

Distortions and Lack of Competition in the Provider Market

Hospitals are the largest and fastest growing 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 2027.⁷² 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. 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.⁷³ Additionally, the analysis found that 320 hospitals – eight percent of those included in the study – marked up some medicine prices more than 1,000 percent. Provider consolidation is another issue which is driven in part by the 340B program, which creates incentives for hospitals to shift care from community-based physicians to higher-cost settings. Congress created the 340B program to assist federal grantees and true safety net 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. At first, grantees made most of the 340B purchases, but over time disproportionate share hospitals (DSH) have come to dominate the program and

account for about 80 percent of 340B sales today.⁷⁴ Current program rules are lax, allowing hospitals to make a profit by dispensing 340B medicines that were obtained at a discounted rate without passing along any of the 340B discount to the patient or using profits to help increase charity care. Hospitals have leveraged their ability to generate revenue from 340B discounts to purchase physician groups.⁷⁵ These shifts in ownership and the site of treatment not only undermine community-based practices but also drive concentration in provider markets, leading to higher prices for payers, the government, and patients.

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.⁷⁶ As a result, insurers pay higher prices for equivalent services that previously were delivered in less-expensive independent physician offices. This hospital consolidation and the resulting mark-ups increase health care costs for patients and providers. Research shows that as hospitals merge, prices increase 20 to 40 percent, with greater price increases in concentrated markets.⁷⁷ As a result, hospital spending accounted for 45.3 percent of 2017 small and individual market premium increases, nearly double any other category.⁷⁸ To encourage provision of health care for patients at lower cost facilities and lower overall costs, we suggest that reforms to the 340B program and incentives which drive excess costs associated with costly provider consolidation.

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 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. Patients with copays pay a fixed amount for each prescription (e.g., \$30), while those with coinsurance pay a percentage of the medication's total list price (e.g., 30%).

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.⁷⁹ Since 2006, deductibles for patients in employer health plans have increased by 300 percent.⁸⁰ Patient out-of-pocket spending on coinsurance has increased 67 percent while spending on copays has decreased.⁸¹ The share of employer health plans requiring a deductible for prescription medicines has more than doubled from 23 percent in 2012 to 52 percent in 2017.⁸² As one recent analysis shows, patients are required to pay 13 percent of overall pharmaceutical costs versus only 3 percent of hospital costs – even though medicine can help keep patients out of the hospital.⁸³

Deductibles and coinsurance 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.⁸⁴ In 2017, more than 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, an increase of 20 percent from 2013.⁸⁵ Patients with chronic conditions are disproportionately impacted by high out-of-pocket costs.⁸⁶

In Medicare Part D, there has been a substantial increase in the use of coinsurance and complex, multi-tiered formularies. Today, 93 percent of stand-alone Part D plans (PDPs) use formularies with five coverage tiers, and 7 percent are now using a sixth tier.⁸⁷ The percentage of Part D drugs subject to coinsurance jumped by nearly 20 percentage points between 2016 and 2019. Today, 62 percent of all medicines covered by PDPs are covered on a coinsurance tier.⁸⁸

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 prescription is

purchased by the patient. These discounted prices are not available to patients with deductibles or coinsurance at the time they fill 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 list prices of brand medicines.⁸⁹ However, since list prices do not reflect rebates, these savings are not directly passed on to patients through lower cost sharing, and patients' out-of-pocket costs for prescriptions filled in the deductible or with coinsurance are higher than they otherwise would be if based on the discounted cost of the medicine. Thus, the growing use of deductibles and coinsurance for medicines has exposed patients to undiscounted list prices and created affordability challenges for many.⁹⁰

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. 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 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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