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Subcommittee on Health

Hearing on 21st Century Cures: Examining the Role of Incentives
in Advancing Treatments and Cures for Patients

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Chairman Pitts, Ranking Member Pallone, and Members of the Subcommittee, I am Scott Hemphill, a Professor of Law at Columbia Law School. I write and teach about the law and economics of innovation and competition. My research has considered the incentives for pharmaceutical innovation and affordable access to drugs established by patent law and drug regulation.[1] I welcome the opportunity to testify today about these issues.

Innovative new drugs have made a major contribution to longer, healthier lives. An innovator's exclusive right to market a new drug is protected by a combination of patents and regulation. This protection furnishes an incentive to innovate, thereby justifying large investments in research and clinical testing. The patent and regulatory systems also serve a second goal, which is to provide low-priced access to life-saving therapies. Robust competition from generic drugs, upon a branded drug's loss of exclusivity, is a powerful driver of lower prices. In 2013, generic drugs accounted for 86 percent of U.S. prescriptions but just 29 percent of drug expenditures.[2] Generic alternatives to branded drugs saved the U.S. health system more than $200 billion in 2012, according to an industry commissioned study.[3]

[1] See, e.g., Paying for Delay: Pharmaceutical Patent Settlement as a Regulatory Design Problem, 81 New York University Law Review 1553 (2006); Earning Exclusivity: Generic Drug Incentives and the Hatch-Waxman Act, 77 Antitrust Law Journal 947 (2011) (with Mark Lemley); When Do Generics Challenge Drug Patents?, 8 Journal of Empirical Legal Studies 613 (2011) (with Bhaven Sampat); Evergreening, Patent Challenges, and Effective Market Life in Pharmaceuticals, 31 Journal of Health Economics 327 (2012) (with Sampat); Drug Patents at the Supreme Court, 339 Science 1386 (2013) (with Sampat).

[2] IMS Institute for Healthcare Informatics, Medicine Use and Shifting Costs of Healthcare: A Review of the Use of Medicines in the United States in 2013, at 30, 40 (2014).

[3] Generic Pharmaceutical Association, Generic Drug Savings in the U.S. (2013).

As an engine of drug innovation, the patent and regulatory systems are not perfectly calibrated. For some innovations, the system confers a windfall. A drug maker invests in the innovation, anticipating a financial reward that is larger than the expenditure even after accounting for the risk of failure. For other innovations, the system may fail to provide a sufficient incentive. A drug maker may judge that the expected returns are not high enough, based on existing protections, and decline to pursue the opportunity. Nor is it the case that the two situations balance out. The windfalls are retained as profit, not spent on developing drugs whose expected returns fail to justify the expense.

Why might the incentive be too small? One possibility is that the drug's development takes so long that little time is left on the patent when the drug is finally approved. Moreover, for some therapies, the active ingredient might be a naturally occurring substance, previously revealed in a scientific paper, or the subject of an earlier patent, making a patent unavailable. The innovator might nevertheless secure a patent on other aspects of the drug, including the active ingredient's use in treating a particular disease. But if this or other patent protection is weak, in the sense that it is judged unlikely to hold up in court, the innovation might be discouraged. A further issue is that for innovative new uses of existing therapies, patent protection might be evaded through off-label generic use, leaving innovators with no practical remedy.

The concern that drug patent protection is inadequate—and that non-patent regulatory protection should be deployed instead—is perhaps surprising; pharmaceuticals are frequently touted as the strongest case for patent protection. But it is not new. It played an important role in the 1984 enactment of the Hatch-Waxman Act and subsequent amendments.[4] These statutes incorporate concerns about inadequate protection by providing special additional protections for drug innovators that are not available to innovators in other industries. For example:

[1] A so-called "new chemical entity" with a novel active ingredient receives five years of regulatory protection from generic competition, after which a generic firm may file paperwork in support of its bid to enter.[5] That process takes some time, so in practice, protection lasts for six or more years.

[4] This testimony focuses on the legal regime for drugs that are chemically synthesized. A full analysis would also examine biologic medicines derived from living sources, which are subject to a different legal regime.

[5] 21 U.S.C. § 355(j)(5)(F)(ii). If a would-be generic entrant challenges one or more branded patents, it may file the paperwork after four years.

[2] If the drug is backed by a patent—even a weak patent—the protection is usually longer, thanks to an automatic stay of generic drug approval while the branded firm sues the generic firm for patent infringement.[6]

[3] Special patent extensions partially compensate for the time spent in clinical trials and the post-trial FDA approval process.[7]

[4] Exclusivity—both regulatory and patent—is extended by six months if the drug maker performs tests to evaluate the drug's pediatric health benefits.[8]

[5] Under the Orphan Drug Act, drugs treating "rare diseases or conditions" receive a seven-year exclusivity period.[9]

[6] The first generic firm to challenge a branded drug's patents is eligible for a 180-day exclusive right to market in competition with the branded firm, before other generic firms may enter.[10] This exclusivity protects the first-filing generic drug maker from entry by other generic firms, and confers a collateral benefit on branded firms by protecting against additional generic challengers until the 180 days have expired.

These additional protections have frequently proved beneficial to innovators in the course of developing new drugs, particularly drugs in which patent protection is otherwise too brief or too weak.

Even with these industry-specific increases in exclusivity, it is likely that some drugs are not developed by drug makers because the rewards are not large enough. The size of this problem in practice is unclear. Assessing the extent of "lost innovation" in the pharmaceutical or any other industry poses a difficult empirical challenge. One careful recent study focuses on clinical trials for cancer, showing that drugs to treat patients with long survival times are disadvantaged by the current system, because the clinical trials are longer, resulting in shorter exclusivity.[11] One question, to which the answer is currently unclear, is whether long clinical trials in general are correlated with more important innovation. Overall, there is a great need

[6] Id. § 355(j)(5)(B)(iii).

[7] 35 U.S.C. § 156.

[8] 21 U.S.C. § 355a.

[9] Id. §§ 360cc.

[10] Id. § 355(j)(5)(B)(iv).

[11] Eric Budish, Benjamin N. Roin & Heidi Williams, Do Fixed Patent Terms Distort Innovation? Evidence from Cancer Clinical Trials (working paper 2013).

for a careful empirical evaluation of the incentives of drug makers, including work that takes an independent look at the internal metrics that drug makers use to assess projects.

To the extent that long clinical trials pose a threat of lost innovation, one option is to alter the structure of trials, by using surrogate endpoints rather than measures of survival, a change that would permit a shorter trial.[12] Another is to fund the trial through a targeted government subsidy, rather than post-approval exclusivity. A third option is a tailored increase in post-approval exclusivity, limited to those types of innovation where the underproduction problem is important.

Section 201 of the MODDERN Cures Act takes a different tack.[13] It offers a large increase in protection for all novel drugs. In particular, it provides a 15-year regulatory term of protection to "dormant therapies" with a novel active ingredient. That term might be taken to suggest a limited scope of application, along the lines of the Orphan Drug Act. But in fact "dormant therapy" is a misnomer. Virtually any drug with a novel active ingredient would receive protection. The key requirement is that a drug must address an "unmet medical need."[14] For example, a disease for which no therapy exists would count.[15] But the standard is extremely elastic, sweeping in drugs that offer a wide range of improved outcomes, different side effects, or even increased "compliance or convenience."[16] It is hard to think of a new chemical entity that would fail this test.

In effect, section 201 extends regulatory protection for new drugs to 15 years. Fifteen years is several years longer than the existing overall protection for most new drugs. In a previous academic study, Bhaven Sampat and I examined a set of 117 drugs with a novel active ingredient that experienced generic entry during the decade between 2001 and 2010.[17] The average (mean) market life for the branded drugs was 12.2 years. The proposed protection is also three years longer than the 12-year data protection for new biologics. It is longer than 10-year (which may be extended to 11-year) data protection in Europe.

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[12] Id. at 15-16.

[13] H.R. 3116, 113th Cong. (2013).

[14] Id. § 201(a)(2)(A).

[15] Id. § 201(i)(1)(A).

[16] Id. § 201(i)(1)(B).

[17] Hemphill and Sampat (2012), *supra*. The paper analyzes 119 drugs with at least one Orange Book-listed patent. Six drugs with no patent protection are omitted. The analysis in the paper includes two drugs that were denied new chemical entity protection because each contained a previously approved active ingredient. Those two drugs have been dropped from the present analysis.

Section 201 would thus grant a windfall for a large number of drugs that would have been developed anyway. As to these drugs, the effect is to transfer money from drug purchasers to branded drug makers. To the extent that patients pay in whole or part for drugs, this provision would also reduce access to existing drugs. Apparently recognizing this issue, an earlier version of the bill made a modest effort to cabin its effect by requiring a showing of "prospectively insufficient patent protection."[18] The provision was quite limited in effect, merely requiring a certification that anticipated post-approval patent protection was less than 14 years. But even that limited provision has been removed from the current bill.

The resulting windfall would be quite large. To obtain a rough estimate, we can examine the 117 drugs discussed above. If all of these drugs were protected instead by a 15-year regulatory term, most would enjoy a multiyear extension of protection; a few would have shorter protection. Taking into account the sales of each drug, a back-of-the-envelope calculation suggests that the switch would transfer $121 billion from purchasers over the course of a decade.[19]

This calculation assumes that all drug makers switch to a 15-year term. If, to the contrary, a drug maker is able to predict when its protection will be longer than 15 years—in other words, when opting into the MODDERN Cures Act would offer less protection than the status quo—it will opt out. In that case, the total transfer would be even larger.[20]

There is a second problem. Fifteen years is likely to serve as a floor, not a ceiling. The 15-year regime appears to be subject to manipulation that has the effect of extending exclusivity. One form of manipulation, to which the MODDERN Cures Act appears particularly vulnerable, is "product hopping." At the end of a branded drug's exclusivity, a branded firm has an incentive to shift patients and doctors to a line extension before generic entry occurs. This shift can be accomplished by promoting the new product, increasing the relative price of the old product, or withdrawing the old product from the market. An example is Namenda, a treatment for

[18] H.R. 3497, 112th Cong. § 201(b)(2)(C), (d)(1) (2011).

[19] The average per-drug increase in branded sales is $2.06 billion (in 2010 dollars), under the assumption that drug sales remain at the same level during the extension or reduction, compared to the benchmark year prior to generic entry, rather than increasing or falling off. Assume further that generic competition would save purchasers one-half of the branded price. Finally, ignore discounting. Applying these assumptions yields a total transfer of $121 billion (= $2.06 billion x 1/2 x 117 drugs) over a decade. This calculation does not include welfare losses caused by price distortions.

[20] For example, 16 drugs in the Hemphill/Sampat sample had a period of exclusivity greater than 15 years. Suppose that the makers of these drugs opted out, and the remaining 101 drugs switched to the 15-year term. In that case, the average per-drug increase is $2.5 billion. Using the same assumptions introduced in footnote 19 yields a total transfer of $126 billion.

Alzheimer's disease. The drug maker has announced that in August 2014, it will discontinue Namenda tablets, thereby assisting its push to switch patients to a newer once-a-day formulation with stronger patent protection. Patients who are doing well with the tablets, and who could otherwise take advantage of a cheaper generic when exclusivity ends in 2015, are deprived of that choice. The absence of protection against product hopping and other tactics would likely extend protection under the MODDERN Cures Act well beyond 15 years.

Finally, the burden of this proposal falls entirely on the shoulders of U.S. purchasers. One consequence is that a particular increase in U.S. exclusivity has a less-than-proportionate effect on drug maker rewards (and hence a lesser effect on incentives), to the extent that the increase occurs in the United States alone. Moreover, U.S. purchasers already bear the greatest part of the burden, through higher drug prices, in supporting innovation that has a global benefit. A further increase in U.S. protection would tend to exacerbate that disparity.

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Claims that larger drug maker rewards would increase innovation are easy to make, but hard to pin down. The right next step is careful study to determine the scope of the lost innovation problem in practice, and if warranted, a solution narrowly targeted at the problem. Targeted solutions that do not confer a windfall include modifications to trial protocols and government support of long-lasting trials where appropriate. Special increases in exclusivity should be narrowly tailored, a concern reflected in the Hatch-Waxman Act and Orphan Drug Act, but missing from the MODDERN Cures Act, which would cost purchasers many billions of dollars in higher prices for drugs that do not require any additional incentive to elicit. Thank you for the opportunity to discuss these issues with the Subcommittee.