The Case for Extending Patent Terms for Drugs that Treat Rare Diseases

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The Case for Extending Patent Terms for Drugs that Treat Rare Diseases

A Thesis

Presented to

the Faculty of Social Sciences

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of the Requirement for the Degree

Master of Arts

by

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Advisor: Dr. Peter Ho
Abstract

This paper seeks to determine if extended patent terms can positively effect the production of drugs that treat rare diseases. It reviews the structure of the modern pharmaceutical company and considers the regulatory history that lead to this structure. The paper applies historically significant economic research in the field of patents and considers patent alternatives to establish the best method for incentivizing the development of these drugs. The paper finds that while there are numerous pitfalls and issues with the patent method, there are no viable alternatives. It also concludes that extending patent terms for drugs that treat rare diseases can act as a catalyst to orphan drug development.
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I. Introduction

Major drug companies generally spend anywhere from US$180 million\(^1\) to US$1.3 billion\(^2\) to research, develop, and prepare a new drug to take to market. Many of the drugs they develop are incredibly important to modern life. Numerous crippling ailments have been either controlled or eliminated all together due to innovations in the pharmaceutical industry. Treatments once available only through expensive surgery and life altering processes are now available in pill form. The patent system was developed to enable and protect these types of innovations by granting the patentee the right to exclude others from making, using, or selling the claimed invention during the life of the patent.\(^3\) Despite this, the patent system, as it relates to pharmaceutical products, is heavily

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criticized for its granting of fixed term monopoly rights to qualified patent holders.

Patents are intended to promote innovation in the pharmaceutical industry by granting limited life monopoly rights for qualified drugs. These monopoly rights provide market exclusivity that enables pharmaceutical companies to recapture the cost of research and development (“R&D”) and facilitate or enlarge their eventual profit. Pharmaceutical companies argue that the magnitude of this potential profit is what induces them, as rational firms, to engage in R&D since there is no guarantee that initial expenditures will lead to a product viable for public consumption. After the R&D process, pharmaceutical companies have to achieve the approval of the Food and Drug Administration (“FDA”) and find a populous to market to. Very few drugs make it through all of these stages of development.

The mechanics of the patent system and the rational behavior of firms result in the production of only economically viable pharmaceutical products. Patented products in high demand are generously rewarded for invention. Rational, profit-seeking firms gravitate towards producing these types of products. There is, however, very little to incentivize inventors of products which

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4 This, of course, ignores the role of government subsidies and programs that are prevalent in some unviable pharmaceutical development programs.
are not in high demand by consumers.\textsuperscript{3} Accordingly, “both impoverished and small disease populations are overlooked because active investment in these groups would not be commercially sound; economic forces favor targeting the afflictions of large and wealthy populations.”\textsuperscript{4} There may be a social need for these items but little to no profit incentive for firms. This creates a void in the current structure of the patent system.

There are several ways in which this void can be addressed, all of which require some kind of incentive for the producer: “Incentives are necessary because market forces will likely drive commercial pharmaceuticals to focus on diseases both dire and profitable, an approach which leaves many other disease groups by the wayside.”\textsuperscript{5} Incentive options include government controlled research and drug development, direct government grants and subsidies, centralized information databanks that can be accessed by all drug developers, or appropriate manipulation of the patent system. This paper postulates that the patent system is more practical to achieve targeted drug research efforts.

\textsuperscript{3} Gifford, Daniel J., \textit{How do the Social Benefits and Costs of the Patent System Stack up in Pharmaceuticals?} 12 J. INTELL. PROP. L. 75, at 82 (Fall 2004).


\textsuperscript{5} Su, supra note 4, at 774.
In order to examine the viability of this approach, this paper is divided into four sections. First, the paper offers requisite background information on the relevant economic aspects of the current pharmaceutical system. Then, the paper critically examines relevant economic theory pertaining to patents. Next, the paper transitions into a discussion on the state of rare diseases and what can be done to incentivize drug development in that realm. Finally, the paper concludes that patents are the best method for incentivizing pharmaceutical companies to extend resources to these concerns.
II. The Pharmaceutical Industry: Some Important Economic Aspects

The structure and operation of pharmaceutical companies and the industry in which they operate are both heavily criticized and controversial. Much of this controversy comes from the process undertaken by firms to develop, market, and profit from their products. This section discusses the nature of pharmaceutical companies and the market in which they operate; the competitive strategies that pharmaceutical companies employ and contend with; the outcome of the industry’s efforts, including prices, profits, and the impact on the consumer; potential alternatives to the patent system; and finally, the section concludes by discussing the evolution of the patent system in the U.S.

A. The Stages in Putting Pharmaceutical Drugs on the Market

1. Research and Development

R&D fundamentally underlies the discovery of virtually all pharmaceutical products. “[I]t now takes an average of 10 to 15 years to bring a
new medicine from the laboratory to the pharmacy.” In 2005, pharmaceutical companies backed a study that purported their R&D cost per approved drug to be around $1.3 billion. Given the time it takes to research and develop drugs and the purported costs associated with that research and development, it is not at all surprising that R&D is the focus of much of the discussion on the pharmaceutical industry.

Chemical compounds called proteins control most human biological functions. Proteins provide structural support for the body: they act as enzymes

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7 Other studies have pinned this timeframe to less than four years. (Donald W. Light and Rebecca Warburton, Demythologizing the High Costs of Pharmaceutical Research, THE LONDON SCHOOL OF ECON. AND POLITICAL SCI. (2011) available at http://www.pharmamyths.net/files/Biosocieties_2011_Myths_of_High_Drug_Research_Costs.pdf.)


9 This figure is the most widely cited industry cost estimate and comes from a study performed by Joseph DiMasi, Ronald Hansen, and Henry Grabowski. However, the fact that this study was backed by the pharmaceutical industry immediately compromises its validity. Furthermore, the study was performed at the Tufts Center for the Study of Drug Development in Boston, which is not only an industry repository, but also receives substantial funding from pharmaceutical companies. A detailed reconstruction of this study found that both the cost of developing a new drug and the associated risks are extremely low up until the large final clinical trials. This reconstruction concluded that pharmaceutical companies generally recovered their investments within the first 18 months of production. (Donald W. Light and Rebecca Warburton, Demythologizing the High Costs of Pharmaceutical Research, THE LONDON SCHOOL OF ECON. AND POLITICAL SCI. 3 (2011) available at http://www.pharmamyths.net/files/Biosocieties_2011_Myths_of_High_Drug_Research_Costs.pdf.)
that accelerate essential chemical reactions in cells; they act as antibodies that protect cells from attack by foreign substances; they even act as metabolic regulating hormones.\textsuperscript{10} Disease causing protein behavior is often attributed to genetic mutations.\textsuperscript{11} Accordingly, genetics is the focus of much of the R&D activities of pharmaceutical companies. Genetic research is also one of the most resource intensive and complex R&D endeavors in human history.

R&D originates in the discovery phase. During this phase, companies investigate chemical compounds and determine their ability to bind to and modify targeted molecules. Targeted molecules are those molecules expected to affect a specific disease. It is estimated that the body contains thousands of these potential targets.\textsuperscript{12} When a promising target is identified, researchers test the target from libraries of molecules developed over the years, largely by a process of trial and error to, determine their likelihood of success against the disease being addressed.

Promising drug candidates are then put through the preclinical research stage. This is where the drugs are tested both in the laboratory and on animals, with a focus on the pharmacological aspects of drug development. The pharmacological aspects of concern include the relationship between dosage and


\textsuperscript{11} Id.

toxicity, bioavailability,\textsuperscript{13} and efficacy. The rate of attrition for compounds at and before this stage is high: Of the 5,000 to 10,000 drug candidates screened during the discovery stage, only about 250 will make it to the preclinical testing stage.\textsuperscript{14}

During the preclinical research stage, drug companies file an investigational new drug (“IND”) application with the FDA, apply for a patent, and start developing economic and quality manufacturing processes for promising compounds. Compounds that successfully complete this stage move onto the clinical trial stage.

Drugs must successfully complete three clinical trials – called phase I, phase II, and phase III – before being considered for market approval. Of the approximately 250 drug candidates that go through the preclinical research stage for a drug development project, usually only 5 make it to the clinical trial stage.\textsuperscript{15}

During phase I testing, the drug is evaluated for safety on healthy individuals. Phase I testing helps determine the maximum safe dose on a particular compound. This is done by increasing the dosage on healthy individuals until side effects are

\begin{footnotesize}
\begin{enumerate}
\item Bioavailability measures the drug’s ability to move through and remain in the body. It looks at the drug’s ability to reach the drug target with sufficient dose for the therapeutic result anticipated. (Aldridge, \textit{Magic Molecules}, 11-12).
\end{enumerate}
\end{footnotesize}
observed. This phase typically involves anywhere from 10 to 100 participants, lasts about a year and a half, and costs about $10 million.\textsuperscript{16}

During phase II, trials are held to establish the parameters for phase III testing of the drug. The parameters to be determined include class (i.e. age and gender) and health of the trial patients, what to quantify, and effective dose and duration of the treatment.\textsuperscript{17} Phase II represents the first instance of control groups taking placebos. An effect from the drug, whether desired or as a negative side effect, must occur in a statistically significant number of participants to be definitive.\textsuperscript{18} The typical duration of phase II trials is about two years, involves 50 to 500 participants, and costs about $20 million.\textsuperscript{19}

Finally, in phase III, quantitative measurements of the effectiveness of the drug are taken along with an evaluation of side effects. This phase of clinical trials is designed to match as closely as possible how the drug would be used in “real life.” This may involve several studies at different locations, each following the same protocol. Patients taking part in these studies are selected based on characteristics defined during the phase II trials. To determine if the drug is pivotal in affecting the disease, a statistically significant portion of the test group

\begin{footnotesize}
\begin{itemize}
\item \textsuperscript{16} Zivan, Justin A., \textit{Understanding Clinical Trials}, 282 SCIENTIFIC AMERICAN 71, at 71 (April 2000).
\item \textsuperscript{17} Rowberg, supra note 15, at 10.
\item \textsuperscript{18} Zivan, supra note 16, at 73.
\item \textsuperscript{19} Id.
\end{itemize}
\end{footnotesize}
must see a measurable improvement over the control group taking the placebo. The FDA usually requires at least two trials concluding that the drug is pivotal to grant its approval.

The clinical trial process is extremely costly. The costs included during this period cover:

- The physicians organizing and running the trials;
- Data verification and analysis;
- Support personnel such as nurses and administrative staff; and
- Payment to physicians and nurses who care for the patients involved.

In addition to the costs referenced above, the research and development stage of drug formulation also includes the cost of capital. Cost of capital here means the opportunity cost of deciding to invest in this particular endeavor as opposed to other opportunities.

2. **Regulatory Approval**

After phase III trials are complete, if the results of the drug testing are positive, a New Drug Approval (“NDA”) application requesting approval to
market the drug is submitted to the FDA. The FDA will approve only those drugs whose results provide a clear indication of effectiveness with manageable side effects. The FDA may even require additional phase III trials if it considers the results of the trials already run inconclusive.

Recent studies have found that it now takes much longer to develop, seek FDA approval of, and produce new useful drugs: “In 2002 the US FDA approved only 17 new molecular entities for sale in the US, which is down from a high of 56 approved in 1996 and is the lowest since 1983.” Furthermore, the amount of time it takes to receive FDA approval for a particular compound is quite significant: In 2003, the average FDA approval time for a new small molecule drug was 16.9 months. The average approval time for a new biologic was 34.7 months.

Once FDA approval is granted, the drug can be marketed to the general populace. However, even after approval, the drug is continually assessed. These assessments are based on the observations of physicians who prescribe the drugs. Drug companies must file adverse drug reaction (“ADR”) reports with the FDA

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20 The requirements of a NDA are discussed in more detail in later sections of this paper.


regularly. In some instances, serious issues reveal themselves in drug use after the FDA has approved the drug. These drugs are then removed from the market.

The FDA may also offer a conditional approval on a particular drug. In this case, the pharmaceutical company may be required to carry out studies regarding safety when the drug is already on the market. These later trials are labeled phase IV trials.

3. The Evolution of the Pharmaceutical Industry and its Patents in the U.S.

The U.S. Constitution states that “The Congress shall have Power . . . To promote the progress of science and the useful arts, by securing for limited times to authors and Inventors the exclusive right to their respective writings and discoveries.”23 The country’s founders thus saw the need to promote innovation in their new land. They also apparently believed that exclusive property rights were the best way to achieve this. Later legislators restricted the breadth of these property rights: 35 U.S.C. §101 requires that an invention be useful, new, and non-obvious to be patentable. These foundational patent references are, however, just the beginning of the story on patent legislation in the United States.

The first notable pharmaceutical patent legislation was the Pure Food and Drug Act of 1906. It significantly preceded the first important groups of anti-

23 U.S. CONST., art. I, § 8, cl. 8.
infective drugs, which were introduced in the mid-1930s. In 1938, in response to the drug disaster that resulted in the death of over one hundred children, Congress passed the Food, Drug, and Cosmetic Act requiring new drugs be approved as safe before being introduced into interstate commerce. The Act was later amended in 1962 by the Kefauver-Harris Amendment requiring that drug efficacy and safety be demonstrated on the basis of well-controlled scientific tests prior to being approved for marketing by the FDA.

The Kefauver-Harris Amendment caused some incongruence between patent law and safety requirements. In response to the Amendment, the United States Patent Office started requiring proof that a compound was safe and effective prior to granting a therapeutic patent. This position was quickly rejected by the Court of Customs and Patent Appeals, noting that the purpose of granting patents was to spur capital investment requisite for developing and marketing inventions regardless of a compound’s compliance with health and safety laws. It then became common practice to file patent applications for


25 Id.

26 Id.


28 Id., at 393.
potentially useful therapeutic compounds prior to safety and efficacy tests in humans.

Subsequent to the Kefauver-Harris Amendment, there was a time of substantial legislative stalemate in regards to pharmaceuticals and the relevant patent process. This period lasted essentially up until the 1980s when public sentiment and therefore legislative policy refocused on pharmaceutical and patent concerns. The Bayh-Dole Act of 1980 was directed towards promoting drug development amongst universities and other federally funded programs. It allowed these institutions to patent and exclusively license their discoveries, thus providing a profit mechanism for their development activities. It has also resulted in the ownership of the intellectual property of new drugs by these institutions.²⁹

Reacting to the limited number of drugs available to treat rare disease, Congress passed the Orphan Drug Act (“ODA”) in 1983. It incentivizes drug manufacturers who specialize in developing drugs that treat rare diseases primarily through a seven-year marketing exclusivity period. During this period, other producers can gain FDA approval for a drug with an identical make-up; however, they are forbidden from marketing that drug. The ODA also provides for a 50 percent tax credit for qualified clinical testing expenses; establishes an orphan drug grant program; exempts qualified developers from FDA “user

fees”; provides a centralized coordination board for qualified producers to reduce wasteful expenditures; provides an assistance program that allows qualified developers to request assistance from the FDA in designing clinical trials; and allows patients to use drugs during the clinical trial phase. All of these provisions enable the drug producer to reduce costs and garner a larger market share. These producers are also allowed to concurrently seek patents on these drugs.

Before 1984, patent law allowed parties to make and use patented products or processes as long as no profit was earned. Patent holders would have to prove that the infringer was deriving benefit at their expense in order to have a legitimate patent violation claim. Based on this reasoning, generic drug companies commonly sought FDA approval to market generic versions of patented drugs before the associated patents expired. Generic drug companies seeking approval to market their products were obligated to file a New Drug

30 While the ODA was enacted in 1983, the FDA user fee was part of legislation that took effect in 1992. Accordingly, this user fee provision was not added to the ODA legislation until after 1992.


32 Engelberg, *supra* note 27, at 394.

33 Engelberg, *supra* note 27, at 394.

34 Engelberg, *supra* note 27, at 395.
Application (“NDA”) and to prove that the compound was safe. This process was done despite the compounds being identical to those previously approved for safety by the patent holder. To facilitate this application, the FDA sometimes accepted published data as proof of the generic compound’s safety; however, the FDA was permitted to request additional expensive clinical trials if there were reports of adverse reactions preceding the its approval of the generic product.

In 1984, Congress established legislation that greatly facilitated competition in the pharmaceutical industry. It enacted the Drug Price Competition and Patent Term Restoration Act of 1984 (“Hatch-Waxman Act”) in response to a 1983 Supreme Court decision ruling that even when patent protection ceased, most generic imitations had to undergo clinical trials nearly as rigorous and costly as the originally approved molecule. Title I of the Hatch-Waxman Act sought to promote the availability of generic drugs while Title II sought to extend the life of drug patents and patents of other regulated products by up to five years. This helped to compensate for regulatory delay and ensure that innovators had “a reasonable opportunity to recoup development costs and to make a profit irrespective of the existence of patents.” The Hatch-Waxman Act also stipulated

35 Engelberg, supra note 27, at 396–97.

36 Engelberg, supra note 27, at 397.

37 Engelberg, supra note 27, at 406.
that the period of post-approval patent exclusivity could not exceed 14 years.\textsuperscript{38}

The Act provided statutory assurances that generic competitors may not enter the market until relevant patent rights had been adjudicated.\textsuperscript{39} Congress also created an exemption that allowed generic companies to use, make, or sell a patented product when related to the development and submission of that product for approval by the FDA.\textsuperscript{40} Finally, the Act required that brand name companies seeking FDA approval for a new drug must file a new drug application ("NDA"). This application must include the patent number(s) and corresponding expiration dates. It also must include information on patents related to methods of using the drug. After the drug is approved, the FDA discloses this list in what is known as the Orange Book.\textsuperscript{41}

Hatch-Waxman overwhelmingly affected how both brand name and generic pharmaceutical companies operated. Prior to its enactment, pharmaceutical companies had to obtain patents on their drugs before applying for the lengthy FDA approval process. This drastically reduced the effective patent term. On the other hand, generic manufacturers were forced to get NDA approval


\textsuperscript{39} Engelberg, \textit{supra} note 43, at 403.


\textsuperscript{41} Oullette, Lisa Larrimore, \textit{Note: How Many Patents Does it Take to Make a Drug? Follow-on Pharmaceutical Patents and University Licensing}, 17 \textit{MICH. TELECOMM. TECH. L. REV.} 299, 304 (Fall 2010).
prior to being able to market a generic version of a drug. This was required despite the drug’s identical molecular composition as a previously approved brand name version. It was also considered an act of infringement to use or manufacture a patented product even if that product was only being tested in preparation for FDA approval.\textsuperscript{42}

To further facilitate competition, the Hatch-Waxman Act modified the FDA approval process to include abbreviated new drug applications (“ANDA”). To benefit from this expedited drug application process, an ANDA must show that the generic drug is already listed, that it has the same active ingredient as the listed drug, that the generic drug will be given in the same manner as the listed drug, and that the generic drug is the listed drug’s bioequivalent.\textsuperscript{43}

Finally, Hatch-Waxman created an “artificial act of infringement” provision. This provision is based upon the submission of an ANDA and allows large pharmaceutical companies to sue generic manufacturers for infringement prior to FDA approval. This is intended to prevent promotion by a generic company of an infringed patent.\textsuperscript{44}

In 1992, Congress enacted the Prescription Drug User Fees Act. It requires drug developers seeking FDA approval to pay a user fee. The FDA agreed to use

\textsuperscript{42} Herlihy, \textit{supra} note 40, at 121.

\textsuperscript{43} Herlihy, \textit{supra} note 40, at 122.

\textsuperscript{44} Herlihy, \textit{supra} note 40, at 122.
the additional revenue associated with this act to hire more reviewers in order to speed up the approval process.\textsuperscript{45} The FDA appears to have followed through on its promise: Since the passage of the act, the time it takes for the FDA to approve a drug has dropped from a 30 month average to an 18 month average.\textsuperscript{46}

Perhaps unsurprisingly, legislation related to patents has created a substantial amount of litigation. Some of the most controversial litigation has revolved around the concept of unapproved use.

"The Warner-Lambert decision has rendered unapproved use patents practically unenforceable by (1) allowing generics to enter the market without any challenge upon the filing of an ANDA and (2) announcing standards for a traditional infringement action that make it nearly impossible to prevent infringement even after the ANDA is approved."\textsuperscript{47}

This issue is exacerbated by the fact that doctors are not regulated as to the designated use of prescription drugs, which enables generic manufacturers to market drugs under a use not covered by patent. "The most important negative impact of this situation is not on the pocketbooks of large pharmaceutical companies. Rather, it is that a brand-name producer will simply not be willing to invest resources for the discovery of new uses."\textsuperscript{48} Warner-Lambert was decided in


\textsuperscript{47} Id. at 134.

\textsuperscript{48} Id. at 135.
2008 and has reduced both the strength of pharmaceutical patents and the incentive for pharmaceutical innovation.

B. Pharmaceutical Patents: Competition Both During and After the Life of the Patent

1. Competitive Practices During the Life of a Patent

One manner by which drug companies attempt to limit pressure from competitors is through broadly defined patent scopes. A broader patent scope allows for greater rights for patent holders. The scope of the patent is determined early on in a drug’s development. In initially seeking patent protection of an apparently beneficial compound, an inventor must first specify to the patent office how the invention works and then describe the claims underlying that specification. For a pharmaceutical invention, an application can make four types of claims: (1) a compound claim covering the chemical entity, including any and all formulations or uses of the chemical entity; (2) a composition claim covering a chemical entity formulated for use as a pharmaceutical; (3) a method-of-use claim covering the use of chemical compound or composition in a specified way; and (4) a process or method of manufacturing claim covering the way in which a compound is produced.

Based on the four types of claims outlined above, the patent rules may provide infringers with incentive to seek judicially determined invalidity for
product uses outside of the scope of the patent claim. The Hatch-Waxman Amendments, discussed in detail in later sections of this paper, make this practice especially pronounced in the pharmaceutical industry since it offers 180 days of exclusivity to the first generic manufacturer that files a new drug application.49 This is because these generic manufacturers could potentially receive a very limited period of market exclusivity simultaneous to the initial patent protection, depending on the scope of that patent: The more limited the scope of the patent’s protection, the more likely it is that a generic manufacturer will contemporaneously be able to profit from the compound.

Conversely, a broad patent scope enables an inventor to apply their pharmaceutical product to many uses. This, of course, widens the potential market for the drug. If the drug can be advertised to this broader market without fear of generic competition, the profit potential of the drug company multiplies. To prevent pharmaceutical companies from taking the pre-emptive strategy of patenting their innovations for every conceivable use, the U.S. Patent and Trademark Office (“US PTO”) limits patent grants to relatively narrowly defined uses.

49 Keith Leffler & Cristofer Leffler, Efficiency Trade-Offs in Patent Litigation Settlements: Analysis Gone Astray? 39 U.S.F. L. Rev. 33, 36 (Fall, 2004) (The limits to competition imposed by valuable patents result in static inefficiency because of consumer welfare losses due to high monopoly prices. These patents, however, are still dynamically efficient since monopoly profits incentivize innovation.).
Pharmaceutical companies may also create drugs known as “me too”
drugs. Me too drugs are simply minor variations of highly profitable drugs
already available. They have become the main output of big drug companies. In
fact, from 1998 through 2003 of the 487 drugs approved by the FDA, 379 (78
percent) were classified as having therapeutic qualities that appear similar to
drugs already available and only 67 (14 percent) were actually new compounds.
This means that firms that hold patents on a particular compound may seek to
introduce more drugs under that patent and increase their revenue gain instead of
finding new drugs to affect a different disorder.

Finally, drug companies that have earned specific patents will often
engage in “penetration pricing.” These companies will price me too drugs
relatively low so as to create a market for those drugs. After this market has been
created, drug companies may increase prices on the drugs in subsequent years
assuming that there is enough market eminence for the drug to continue to be
profitable.

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50 Angell, Marcia, *Excess in the Pharmaceutical Industry*, 171:12 CANADIAN MEDIC.

51 *U.S. Food and Drug Administration Center for Drug Evaluation and Research, 
Department of Health and Human Services. NDAs Approved in Calendar Years 1990-
2003 by Therapeutic Potential and Chemical Types (Jan 21, 2004).

52 ZJ Lu & WS Comanor, *Strategic Pricing of New Pharmaceuticals*, REV ECON STAT.
80, 108–18 (1998); Reekie, WD, *Price and Quality Competition in the United States 
2. Post Patent Competition

Perhaps the most salient argument against patents is that when they expire, optimal use of the product they protect is more readily achieved. The effective life of a pharmaceutical patent is currently estimated to be between about eight and twelve years, with the range narrowing as compounds get more complex. Once the patent expires, generic pharmaceutical companies replicate the drug’s chemical make-up at a much lower cost since they forego the sunk costs of initial research and development. The prices of products previously under patent protection therefore decline due to increased competition: “[B]y 1996, forty-three percent of the prescription drugs sold in the United States were generic, as compared to just nineteen percent in 1984.”

More recent figures show that generic pharmaceuticals continue to proliferate the drug market. In 2004, generic pharmaceuticals composed 56.4 percent of the market for drugs: By 2009, generic pharmaceuticals composed 74.5 percent of the market share. The gains in market share of generic pharmaceutical companies are due in large part to the numerous drugs introduced to the market during the 1990s that went off patent during the 2000s. It resulted in


an increase in the number of drugs susceptible to generic competition from 65 percent in 2003 to 81 percent in 2009.\textsuperscript{55}

Logic would suggest that introducing generic competition to pharmaceutical markets would result in price declines on drugs. As generic competition gains more and more market share, the average pharmaceutical treatment cost has decreased significantly. These costs have decreased across all therapeutic treatment classes by a weighted mean of 35.1 percent.\textsuperscript{56} Accordingly, in the case of generic drug proliferation, reality follows logic.

Brand loyalty does cause some consumers to remain with the previously patented drug. This brand loyalty may be on the part of the consumer who used the drug before it was available off patent. It may also be on the part of the doctor who is familiar with the previously patented drug and/or its sales representatives. Marketing by big pharmaceutical companies may also cause doctors and patients to continue to purchase certain brand-name drugs. However, outside of these limited circumstances, pharmaceutical companies that previously enjoyed patent protection can expect to lose a very large portion of their market share at the expiration of their patent.

\textsuperscript{55} Id. at 6

\textsuperscript{56} Id. at 20.
C. Market Outcomes

1. Prices for Drugs with Patent Protection

The U.S. provides a useful case study to determine the affect of patents on the price of prescription drugs. This is because the U.S. employs very little regulation of prices in the presence of patents and adheres to the patent standard for the protection of inventors’ property rights. Exacerbating this issue is the fact that there are more pharmaceutical lobbyists in Washington D.C. than actual Congressmen.57 These lobbyists have been able to effectively in continue, and sometimes extend, patent protections for drugs.

Brand-name prescription drugs in the U.S. are 35 percent to 55 percent higher when compared to other industrialized countries nations.58 With this disparity in pricing in mind, Patricia M. Danzon and Michael F. Furukawa compared drug prices in nine countries with different regulatory structures to determine whether U.S. consumers were disadvantaged by the current structure. They found that the U.S. had the second highest drug prices for both patented and


non-patented drugs and Canada (a country that highly regulates drug prices) had the lowest prices.\textsuperscript{59}

One of the most important conclusions for Danzon and Furukawa’s study was the pricing in the U.S. of drugs still on patent. While other countries do allow for patent protection of certain pharmaceuticals, they strictly regulate that pricing. Because of this, U.S. drug prices are the second highest in the world for drugs still on patent. However, over-the-counter products are considerably cheaper in the U.S. when compared to other countries.\textsuperscript{60}

Danzon and Furukawa took their study one step further to view the results in terms of real dollars. They found that deflating the data by using health purchasing power parities, all other countries have higher drug prices than the U.S., with the exception of France who is on par with the U.S.\textsuperscript{61} What this means is Danzon and Furukawa viewed the price of drugs in the U.S. relative to the price of all healthcare products and services. They found that prices for \textit{all} health services in the U.S. are higher than other countries, not just drugs. High prices for drugs in the U.S., therefore, are emblematic of the high price of healthcare in the U.S.

\textsuperscript{59} Patricia M. Danzon and Michael F. Furukawa, Prices and Availability of Pharmaceuticals: Evidence From Nine Countries, Health Affairs, W3-526–W3-527 (October 29, 2003).

\textsuperscript{60} \textit{Id.} at W-3 527–30.

\textsuperscript{61} \textit{Id.}
Price increases for pharmaceutical products in the U.S. have historically been greater than contemporaneous increases in other products. From December 2008 through December 2009, the producer price index for pharmaceutical and medicine manufacturing increased by almost 6 percent.\textsuperscript{62} Over the same time period, the producer price index for total manufacturing industries increased by 4 percent and that for total wholesale trade industries decreased by 0.1 percent.\textsuperscript{63} The effect of this data is even greater when noting that this was a period of economic contraction.

2. \textbf{Industry Profits}

In 2002, the median profit margin of the top 10 drug companies in the U.S. was 17 percent, which is significantly higher than the 3.1 percent median profit margin earned for all other industries on the Fortune 500 list.\textsuperscript{64} Broken down into revenue terms, drug manufacturers on average received $0.74 on every dollar spent by consumers on prescription drugs going into 2000.\textsuperscript{65} Extremely high profit margins are generally associated with high-risk endeavors such as start-up companies. However, the risks associated with pharmaceutical companies

\begin{itemize}
\item \textsuperscript{63} Id.
\item \textsuperscript{65} Kaiser Family Foundation, \textit{Prescription Drug Trends}, Fig. 3.2.
\end{itemize}
have drastically decreased thanks to technological innovations. Pharmaceutical companies are therefore receiving profits in excess of the risk they are actually incurring.

High revenue figures and profit margins may be explained, at least in part, by the fact that profits relate to all drugs owned and introduced by a specific pharmaceutical company, many of which have existed for some time. While some “blockbuster” drugs will more than just appropriate the relevant R&D costs, most drugs are wholly unsuccessful in economic terms. The likelihood of a compound in preclinical development actually making it to the market is less than 1 in 4,000. Only three out of every ten prescription drugs actually taken to the market will produce enough revenue to recoup costs spent on R&D. Pharmaceutical companies will often require one blockbuster drug to compensate for the numerous commercially unviable drugs. Thus, the amount of time that a pharmaceutical company has existed and its number of previous successes are highly determinative in its profitability.

“Pharmaceutical companies are extremely profitable, because they are already in a mature state of an R&D company, i.e. they have projects in each stage of the life cycle and a renewing portfolio . . . Because of the small probability and the long development time profits on drugs,


and consequently their price, must be high. Otherwise innovation would not pay off.”

The aggregation of the company’s successes and failures facilitate the overall profitability of the company despite numerous commercially unviable drugs. The proper manner in which to characterize the profitability of a pharmaceutical company therefore depends on the specific slice of the company in view.

As with any industry, it is important to consider the mechanism of economies of scale as they relate to the pharmaceutical industry. This mechanism is especially important when considering the profitability of drug companies that operate in countries with price controls. When drug companies expand their markets by entering other countries, they are able to reduce the unit costs of production. They can then invoke Ramsey pricing, which is a process by which companies vary the prices of their goods depending on the elasticity of demand in the market they are entering. When firms enter countries with price controls, they will continue to sell in those countries despite the elimination of monopoly rents because they will still earn normal returns to their capital. Under the Ramsay pricing model, these firms will still charge the higher prices in the non-price controlled countries, enabling them to continue to reap above normal profits.

68 Why Drug Prices Must be High, 11 NEWS IN ADVANCE: VALUATION IN LIFE SCIENCES (March 2010).


70 Id. at 1217.
Expanding on the concept of price regulation and its effect on pharmaceutical profits, John Vernon composed a study to determine the magnitude of this effect. As previously discussed, many countries outside of the U.S. have some kind of drug price regulation system. This may be in the form of direct price controls, which is the case in France and Italy, limits on social insurance reimbursement, which is the case in Germany and Japan, and firm profit controls, as in the United Kingdom. Vernon narrowed his analysis to the top 20 pharmaceutical companies in the world. Of these 20 firms, only 14 firms had sufficient data available to perform a regression analysis. Of these 14 firms, only nine of them did not undergo a merger during the review period. His period for review was from 1994 through 1999. Vernon found that while pharmaceutical companies operating in countries with price controls are still profitable, they have considerably lower pre-tax pharmaceutical profit margins.\(^{71}\)

Vernon’s findings seem consistent with expectations on drug company profitability across different nations. That being said, variable profit margins may depend on factors other than just regulation. There is a “systematic difference in the types of pharmaceutical products sold in the USA relative to the rest of the world.”\(^{72}\) The U.S. is known for demanding drugs that treat things that simply make life more enjoyable (e.g. Viagra or mood enhancers). Medical practices that


\(^{72}\) Id.
offer substitutes to drugs may vary from one country to the next. The cost of a hospital stay or even the cost to visit a doctor can also affect demand for drugs. These factors, among others, will work to limit or exacerbate the effect of price controls on pharmaceutical profits. Accordingly, while Vernon found that pharmaceutical companies are less profitable in countries with price controls, there are many other factors at play in this finding.

D. Some Simple Numerical Illustrations of the Aforementioned Aspects

The following sections apply much of the previous discussion to pharmaceutical development figures available in the public domain. The first section looks at the process that a pharmaceutical company goes through when deciding to develop a new drug. The second section applies three frameworks for analysis of a second tier of patents that extends their life in the case of orphan drugs.

1. Considerations of the Term Variation

Under typical circumstances, when deciding whether or not to invest time and resources to develop a new drug, pharmaceutical companies perform a cost/benefit analysis. This cost benefit analysis weighs the anticipated costs of the endeavor against anticipated benefits of developing the pharmaceutical. Pharmaceutical companies therefore look to the success rates in general for
developing a new drug, netted against economies of scale the company has been able to develop. They also take into account any other possible facilitating factors, such as experience and previous known breakthroughs.

In developing their cost/benefit analyses, pharmaceutical companies first estimate the likelihood of successfully developing a new molecular formula that could be useful to human beings. This likelihood obviously varies on the type of drug and the amount of existing research. As noted in prior sections, of 5,000 to 10,000 initially identified drug candidates, only 250 will, on average, make it to preclinical testing. That is, only 2.5 percent to 5.0 percent of identified drug candidates make it out of the initial exploratory stage. For simplicity’s sake, this analysis assumes that the drug company is experienced in the product and benefits from economies of scale in development on the particular drug it is attempting to develop: Therefore estimated likely success rate is assumed to be 50 percent.

Next, the pharmaceutical company must consider the likelihood that its new drug will achieve FDA approval. As also previously referenced, FDA approval occurs for only about 1 percent of new molecular formulas. Applying this 1 percent approval rate to the 50 percent likely success rate for developing a drug that will make it through preclinical testing, the overall probability of

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success for this particular firm on this particular drug is thus 0.5 percent (50 percent * 1 percent).

After determining the overall likely success rate of the drug, the pharmaceutical company must consider the profit it expects to earn on the new drug once it reaches the market. Since the probability of success is extremely low, a rational firm will require a high potential return on investment to justify the very high likelihood of failure. The potential profits of the drug must also take into account the sunk costs of research and development as well as the discount rate. Ignoring, for the time being, the large research and development cost base, assume that the expected profit on the new drug is $1 million per year. This would garner $11 million based on the average effective patent life of prescription medications. Assuming that the firm recognizes earnings at the end of each of the 11 years of the effective patent period, the $11 million would be discounted in year one so that $1,000,000/(1+r), where r is the interest rate. The $11 million will be discounted for years one and two in the second year: $1,000,000/(1+r)^2. The present value over the course of the patent’s effective term would thus be:

\[
\sum_{n=1}^{11} \frac{1,000,000}{(1+r)^n}.
\]

While patents have a total life of 20 years, the effective patent life of prescription drugs is only 11 to 12 years. Herlihy, Reid, Note: The Federal Circuit’s Interpretation of the Hatch-Waxman Act: Allowing Generics to Induce Infringement, 15 FED. CIR. B.J. 119, at 133 (2005/2006).
At an interest rate of 5 percent, the $11 million in future profit would have a net present value of $8,306,414.22. If the cost of research and development for this drug were a modest $5 million, the net present value of the proposed drug would be further reduced to $3,306,414.22 over the 11-year effective patent life. Based on the risk analysis associated with this particular drug, this firm would have a 0.5 percent chance of earning $300,583.11 per year for 11 years, which converts to a total expected return over the life of the patent of $41,532.07.

If the effective patent life were extended from 11 years to 20 years (i.e. no time is lost for research and development or regulatory approval), the firm would see higher returns overall and on an annual basis. The net present value of the potential profits, taking into account sunken research and development costs would increase to $12,462,210.34. This converts to an annual profit of $373,110.52 over the effective life of the patent. Accordingly, in this scenario the firm now has a 0.5 percent chance of earning $373,110.52 per year for 20 years, which is a total of $12,462,210.34. The firm therefore has a total expected return of $62,311.05 on this particular investment (a 50 percent increase). It is important to note that in this example, 20 years constitutes the maximum potential annual profit: After this point, while profits continue to increase, profits per year decline.

If the risk factor were to change from 0.5 percent chance of success to one percent chance, the firms expected return would of course double. What this demonstrates is that the higher the risk related to developing a new drug, the
larger the potential profit figure would have to be to justify undertaking that risk. It shows that patent terms don’t have to be extended themselves to upwardly adjust the profit potential on a drug: Rather, the effective patent term can be extended simply by reducing regulatory approval time and/or compensating for time lost to development. This would increase the profit potential by extending a firm’s monopoly reign.

2. **Incentivizing Development for Orphan Drugs by Formulating an Optimal Patent Term**

Building on the hypothetical comparison described above, it is useful to consider the implications of extending patent terms for drugs that treat rare diseases. Turning to Daniel J. Gifford’s framework, consider the implication of various patent lengths. Presumably, some inventions would be produced without any type of patent coverage, while some inventions would not be produced even with a full twenty years of exclusivity. Also, presumably, each year of the patent term incrementally stimulates more invention. Finally, under this framework, invention is measured in the value to the marketplace.

In his model, Gifford recognizes that because many inventions are afforded patent protection, they are not sold into restrictive markets. While acknowledging that these inventions may offer some social benefit, Gifford concludes that the restrictions on market access for the invention limits the invention’s overall social value. He therefore reduces the social value earned by
these inventions to 75 percent of their value in an unrestricted market. The social loss of the patent is assumed to be the remaining 25 percent that could be earned in a competitive market.\textsuperscript{75}

Gifford’s analysis can be viewed from two perspectives. It can be looked at in a glass is half empty context, meaning that 25 percent of potential beneficiaries are excluded because of the restrictive patent market. It can also be looked at from a glass is half full perspective because 75 percent of social gain would not have occurred if not for the invention. The issue then is whether or not this invention would have occurred without the restricted market. Furthermore, if some inventions may occur without the restricted market and some will not, how does a society successfully incentivize both inventions while minimizing potential social loss?

In answering the questions posed above, Gifford concludes that a patent system that establishes only one term length for all inventions has the effect of increasing marginal social costs for each superfluous year of patent protection. In other words, the one size fits all patent term length creates excessive marginal social costs to society. That is because market restrictions apply to products for periods beyond what would be necessary to compel the inventor to invent.\textsuperscript{76}

However, according to Gifford, extended patent protection for products that need

\textsuperscript{75} Gifford, Daniel J., \textit{How do the Social Benefits and Costs of the Patent System Stack up in Pharmaceuticals?} 12 J. INTELL. PROP. L. 75, at 99 (Fall 2004).

\textsuperscript{76} Gifford, \textit{supra} note 77, at 103.
it to incentivize their development generates increased marginal benefits on those products. Weighing these marginal benefits against the marginal cost associated with the patent protection helps determine the optimal patent length for a particular product.

Amir Khoury considers the optimum patent protection to be the point where:

“[T]he level of patent protection still provides ample incentive for continued research and development by the patentee while not excluding innovative newcomers or hampering consumers’ access to innovation.”

He notes that this point varies and lists what he considers the determining factors. Specifically:

1. The ratio between the conventional patent term and the projected scientific relevance of the product.

2. The ratio between the successful patents in that field and the aborted research in that industry.

3. The time needed to reap profits to cover the R&D.

4. The cost of the investment that is required to bring the patented product into commercial application.

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5. The time that is needed to create the invention.

6. The time that is needed to reach commercialization.

7. The scope of the market.\textsuperscript{78}

As Khoury notes, these considerations are especially relevant to the pharmaceutical sector. This is because for some drugs, the scientific relevance might be great, which supports increasing patent protection under Khoury’s first factor. There is also a high rate of aborted research in the pharmaceutical industry, again supporting extended patent terms under Khoury’s factors. In fact, each of Khoury’s elements applies to the pharmaceutical industry and, in most cases, help formulate the argument for increased patent protection relative to the protection offered for other products.

Applying Khoury’s factors to the pharmaceutical industry supports the notion that some patent terms should be extended in order to promote innovation. Khoury did not, however, contemplate how this argument is exacerbated when considering rare diseases. The case for extending patent terms for rare diseases is supported most directly by Khoury’s final element, the scope of the market. If, as Khoury stipulates, patent lives should be extended in the case of many pharmaceuticals, then it is reasonable to believe that further extension would be

\textsuperscript{78} Khoury, \textit{supra} note 79, at 409.
justified for drugs with a more limited market scope, as is the case with drugs that treat rare diseases.

To further bolster the contention that extended patent terms can help incentivize research and development in certain markets, the analysis turns to the health production model. The health production model is a useful tool in demonstrating how an extended patent term will facilitate drug development for certain disenfranchised groups. The model links entry, revenues, R&D, new drugs, health, and consumer surplus in a logical sequence.\textsuperscript{79} The specified links include:

1. Entry causes revenue to fall: $TR = TR(E)$

2. Declines in revenues lead to declines in R&D: $RD = RD(TR)$

3. Declines in R&D lead to declines in the number of new chemical entities: $NCE = NCE(RD)$

4. Declines in the number of new chemical entities lead to declines in human longevity: $LY = LY(NCE)$

5. Declines in human longevity lead to declines in welfare/consumer surplus: \( W = W(LY) \).\(^{80}\)

The model shows that increased market competition for a particular drug reduces the revenue earned by the inventor of that drug. Applying this case to a situation in which expected revenues are likely to be small due to a limited market scope exacerbates the subsequent steps. Consider the model in the framework of orphan drugs where the affected population is less than 200,000 people. The average price of a branded pharmaceutical product in 2007 was $119.51.\(^{81}\) This puts static total revenue (TR) for this drug at $23,902,000. Now consider the effects of competition on this drug based on the health production model.

1. \( TR = 23,902,000 \times 35\% = 8,365,700.00 \). According to a study performed by Grabowski and Vernon, the long run effect of generic entry branded drug manufacturers is a decline in revenue of 65 percent.\(^{82}\)

\(^{80}\) Hughes, Moore, & Snyder, supra note 81, at 36–37.


2. RD = $8,365,700.00 (35%) = $2,927,995.00. A PriceWaterhouse Cooper’s report found that a 65 percent decline in revenues leads to a 65 percent decline in research and development spending.\(^83\)

3. NCE = 35\%($2,927,995.00) = $1,024,798.25. According to a study performed by Jensen, a 65 percent decline in research and development spending leads to a 65 percent decline in new chemical entities.\(^84\)

4. LY = 1,021.53. Lichtenberg found that a 65 percent decline in new chemical entities reduces longevity by 1.6 million life years per year.\(^85\) Since we are considering a limited population of only 200,000, the 1.6 million life years figure was adjusted down based on the total U.S. population to come-up with a conservative estimate of the life years lost.\(^86\)


\(^{84}\) See, Jensen, Elizabeth J., Research Expenditures and the Discovery of New Drugs, 36 THE JOURNAL OF INDUSTRIAL ECONOMICS 1 (September 1987).


\(^{86}\) This involved a simple adjustment of dividing the life year figure by the population percentage under consideration, i.e. LY = 200,000/Total U.S. Population*1,600,000.
5. \( W = 1,532,290,924.34 \). Topel and Murphy found that the value of the longevity decline calculated by Lichtenberg is $240 billion.\(^87\)

This figure was reduced in the same manner as the longevity decline by adjusting downward based on the total U.S. population.

The health production model shows the extent of life years lost, as well as the value of those life years, due to competitive pressures in the orphan drug industry for rare diseases. It is important to bear in mind that the health production model is a one-sided analysis that does not consider welfare losses due to consumers being priced out of the market. It does, however, emphasize the fact that most of these drugs are not being developed without adequate incentives. Under normal conditions, patents are granted for a limited term that is hopefully long enough for producers to recapture a sufficient profit for time and effort in development. When potential revenues are reduced due to a small population target, the likelihood of receiving sufficient profit is drastically reduced. Thus, consider the health production model described above but without the generic entrance.\(^88\)

1. \( TR = $23,902,000.00 \)
2. \( RD = $8,365,700.00 \)
3. \( NCE = $2,927,995.00 \)

\(^87\) Kevin M Murphy & Topel, Robert H., *The Value of Health and Longevity*, 114(5) JOURNAL OF POLITICAL ECONOMY 871 (October 2006).

\(^88\) This paper is not proposing no generic entrance in these markets: This model demonstration is purely for comparison purposes.
4. \( LY = 0 \) decline

5. \( W = 0 \) decline

While this paper is not advocating zero generic entrance into these markets, the above comparison expresses the stark difference between a competitive market for these drugs and a patent protected market. It can thus be argued that allowing continued monopoly profits for a time period beyond the current effective life of patents would facilitate development drugs treating rare diseases.
III. The Economics of Patents

The following sections delve more deeply into the economics of patents. They review the concept of public goods, which is generally the driving force and justification of the patent system. The paper then goes on to discuss some of the contributions made by various scholars in this regard. Finally, there is a section reviewing theories and justifications of patent recalibration because a recalibration of the system is what this paper inevitably advocates.

A. The Public Goods Feature of Ideas and Knowledge

Within the framework of the pharmaceutical producer lie two competing interests: The drug manufacturer and the drug developer. In 1961, Kenneth Arrow analyzed these competing interests. Arrow identified a paradox that stymied the free flow of information between resource strapped inventors (in this case the drug developers) and idea starved producers (the pharmaceutical manufacturers). He noted that the solution to this paradox was to enable property rights to inventors of non-rivalorous goods. \(^{89}\) Without these property rights, inventors

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would be unlikely to disclose full details of their invention, limiting the likelihood of public dissemination of their potentially beneficial idea. This is because, as Arrow identified, inventors face the issue of public goods.

Public goods are a specific type of good. They are those goods that are: (1) not depleted when shared; (2) do not allow for exclusion once the good is created; and (3) have very little incremental cost for additional users. Public goods, therefore, are inherently unprofitable.

Drugs are public goods because they are cheap and easy for a competitor to copy. This potential for cheap copying: (1) limits depletion of the drug because they can be produced easily; (2) does not allow for exclusion because pharmacists and other manufacturers can create the drug at their will; and (3) the incremental costs are low without the cost of research, development, and regulatory approval. Pharmaceuticals are therefore public goods.

Based on the traits listed above, private markets may fail to produce public goods at their socially optimal level. Under normal market conditions, competitive conditions force companies to lower prices until they reach their marginal cost. This generally creates a socially desirable price level, at least in the short-run. However, in cases of high fixed costs or high costs sunk into initial R&D, this may result in innovating firm insolvency. Knowing this inevitability, potential inventors of public goods are unlikely to engage in innovation. Under
these circumstances, inventors in an unregulated free market are unlikely to
devote an optimal amount of capital to innovation.90

While Arrow advocated property rights be applied to public goods, there
are certain issues that go along with this methodology. Because patent property
rights create monopoly markets, the market price will not be Pareto Optimal and
there will be some amount of deadweight loss in the product market. The patent,
therefore, creates market inefficiencies. These inefficiencies are not eliminated
until the market is allowed to act competitively after the patent expires.

The use of patents to overcome the public goods problem also creates
monopoly rents, which may lead to rent seeking behavior. Monopoly rent occurs
when products are sold at prices above their actual value. In the case of
pharmaceuticals under patent, because the patent holder has exclusive control of
the sale of the product, the patent holder can set its own price. While this practice
may price some consumers out of the market, the excessive profits earned more
than compensate for the loss of potential end users. Producers therefore have
incentive to charge prices well above the competitive market level.

Excessive profits from patent derived monopolies compel rent-seeking
behavior. This is evident in the pharmaceutical industry, which has more lobbyists

90 See Bambauer, Derek, Legal Response to the Challenges of Sports Patents, 18
HARVARD J.L. & TECH. 401, 413 (2005); Chien, Colleen, Cheap Drugs at What Price to
Innovation: Does the Compulsory Licensing of Pharmaceuticals Hurt Innovation?, 18
in Washington D.C. than any other industry and, with 1,507 lobbyists in 2012, more lobbyists than Congressmen.\textsuperscript{91} Rent-seeking behavior has the unfortunate effect of limiting competition in the industry by imposing artificial barriers to entry such as complex or expensive regulations. Such behavior results in a misallocation of resources for companies so that they no longer focus on improving their product but rather spend time, money, and energy on lobbying efforts. It also limits industry competition and diversity by making it difficult for new entrants. As noted by Mancur Olson, the more a country or industry is dominated by interest groups, the less economic vitality exists and the more likely it is for the country to fall into decline.

Overcoming the public goods problem with patent based property rights may also bottleneck continued development. For example, if Firm A invents a new drug and patents that drug, society is theoretically benefitted because people can now use the drug and the invention can form the basis for future improvements. However, if the drug is patented, future improvements developed by Firm B cannot be researched and marketed until after the patent’s expiration. Firm A does not have any reason to improve on the product because they are already guaranteed monopoly prices. This will retard development by at least the life of the patent. Compounding this over the life cycle of each major

development in the pharmaceutical industry can put society behind in its
development by an unimaginable factor.

Given the negative implications of solving the public goods problem via
property rights, there are other possible modes of incentivizing innovation. One
such solution involves a unifying agency, likely organized by a central
government, that provides a manner in which those who benefit from the good
cooperate with each other so as to pool resources. In the instance of
pharmaceuticals, the pooling of resources could be through a central database of
research results, thereby reducing the cost of future research and development.
However, a cost reduction regarding data collection does not reduce the costs
associated with regulatory approval. It also does not overcome the risk that the
drug may never reach the market or be profitable enough to compensate for the
initial sunk costs.

Another possible solution to the public goods problem in regards to
pharmaceuticals is more direct government involvement. For example,
government may simply step in and provide the public good and/or tax for its
provision. Government may also subsidize the production of specific drugs, which
would enable some semblance of a competitive market for end-users. The concern
for both of these solutions is that they involve ex-ante decisions as to both funding
size and treated diseases. Furthermore, both of these solutions rely on the
government’s general fund, which is sourced from taxes. Accordingly, the cost of
these programs is not limited to only benefitted individuals, but rather is spread across all groups. Tax based programs are of further concern when recognizing that Congress controls the “purse strings.” That is, funding may be cut by a Congressional whim, dissatisfaction of constituents, or through spending focused austerity measures.

As outlined by this section, the public goods problem for invention has several potential solutions. Kenneth Arrow advocated the use of property rights to enable inventors to reap the benefits of their innovation, although, as the next section discusses, Arrow had many concerns regarding the monopoly structure. There are alternatives to property rights that can offer a solution to the public goods problem. Many of these alternatives require an omniscient presence with ex-ante decision capacity (i.e. central government). The major issue with these solutions, beyond the forward-looking decision-making, is the funding source. Patents, therefore, appear to the best manner in which to overcome this public goods problem, at least based on the tools presently available.
B. Do Patents and the Monopoly Structure Induce or Stifle Innovation?

1. The Perils of the Monopoly Structure - Kenneth Arrow

Kenneth Arrow believed that “the incentive to invent is less under monopolistic than under competitive conditions.” This is because new inventions cause old technology to become obsolete, causing the monopolist to have a “strong disincentive for further innovation.” Essentially, if a firm does not face competitive market pressure it will stagnate in its innovative endeavors. Arrow argues that this concept is particularly relevant where there are drastic innovations and post-invention monopoly prices are less than the pre-invention monopolist’s costs. This structure is exemplified in markets where patent terms outlast the requisite length to induce innovation.

Arrow acknowledges that gaining market efficiency is not a “one-size-fits-all” proposition. He states that the determination of optimal resource allocation for invention depends wholly on the type of invention and the market for knowledge. In this context, Arrow points out that competition insures a Pareto optimum result under certain hypotheses. The basic conditions for this potentially

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93 Arrow, supra note 137, at 158.

94 Arrow, supra note 137, at 157.
Pareto optimal result is: (1) well-defined utility functions for consumers and transformation functions for producers; and (2) the transformation functions do not display indivisibilities.\(^{95}\) Focusing on the first condition, two assumptions must be met to reach Pareto optimality: (1) No uncertainty in the production relations and utility functions; and (2) all relevant commodities must be traded on the market. In order for these two assumptions to be met, the commodity must be made into private property.

Arrow’s analysis transitions to a comment on the state of patents when he considers the situation of information as a commodity. The cost of transmitting information is very low. If this cost were, in fact, zero (which arguably it is in the internet age), then the optimal allocation of this information would be unlimited distribution without cost. Furthermore, information is completely indivisible. The indivisibility of information combined with its optimal allocation results in a difficult model for profitability.

The information’s owner is in an unusual predicament. He cannot simply sell the information on the open market because it would be impossible for him to preclude others from using it. If he creates a monopoly and uses the information himself, he will not be able to exploit the value of the information for any type of monetary gain. Legally imposed property rights, such as patents, are a manner in which the possessor of information can appropriate value from the information.

\(^{95}\) Arrow, supra note 137, at 157.
Risk also plays a factor in considering resource allocation of patentable inventions. The inability to bear risk can give rise to suboptimal allocations of resources. That is because firms cannot measure their expected output relative to their current inputs regarding a specific endeavor: Firms do not know if their investments are likely to prove valuable. According to Arrow, the current economic system does have devices for shifting risk, but they are limited and imperfect.

Arrow states that in a free enterprise economy, in order for invention to be profitable, there must be a suboptimal allocation of resources. This is because, as discussed previously, the cost of transmitting the knowledge gained from inventive activity is zero. Accordingly, any price for that knowledge above zero is suboptimal. Patents ensure this suboptimal result.

Patents exist to help inventors appropriate the information they produce. Arrow postulates that in order to create any type of efficiency, patent laws would need to be incredibly complex and subtle to allow large-scale appropriation. In order for an inventor to appropriate his knowledge, if he discovers something, any user of that discovery must pay for it. It doesn’t stop there though. If that inventor somehow sparks an idea for another inventor, who bases his new invention on the original inventor’s knowledge, the new inventor must also pay for use of the original inventor’s information. According to Arrow, under this system, “[o]ne would have to have elaborate distinctions of partial property rights of all degrees”
to make it work efficiently. This is because information, which is already difficult to quantify, is not only the output of inventive activity, it is also the input of inventive activity.

The nature of information as both an input and output of inventive activity is where the patent issue lies. By creating property rights for patent holders, patents preclude other inventors from building on existing information. Inventive activity therefore stagnates because patent holders have no incentive to improve their existing and profitable technology. New inventors have incentive not to use readily available knowledge for fear of being sued. Patents therefore, according to Arrow, stifle innovation.


Boldrin and Levine argue that there is no empirical evidence to suggest that patents increase either innovation or productivity. They state that the patent system’s basic problem is that it blocks future innovation with existing monopoly grants. Boldrin and Levine illustrate this problem with the current relationship between Microsoft and Motorola. Microsoft holds a patent for an application used on the Android operating system. Motorola must pay a fee to license the use of

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96 Arrow, supra note 137, at 157.

this idea on its smart phones. In addition to the Microsoft patent, there are many other patented ideas that are used on smart phones, all of which must be compensated for by Motorola. Dealing with these license fees creates a “hold-up” problem for Motorola: That is, in creating a new smart phone, Motorola must purchase many licenses, which compromises the ultimate value of the new phone. Motorola is therefore less likely to develop a new smart phone.

Boldrin and Levine also argue that, counter to logic of patent proponents, there is no empirical evidence to suggest that patents enable the sharing of ideas and limit secrecy.\(^98\) They use the example of open source software, which is software with coding available to all to use and modify. Open software is copyrighted and valuable to the original developer; however, the original developer does not exclude others from using and enhancing his innovation. Boldrin and Levine compare this model to that of Microsoft, with its infamous software and operating system disasters. Had Microsoft had a less secretive approach to Windows Vista, perhaps the operating system coding issues and bugs could have been fixed.

Applying their theories to the pharmaceutical industry, Boldrin and Levine propose reducing drug maker profits to a competitive 5 percent of the cost of drugs.\(^99\) They argue that this would reduce overall drug prices by about 50 percent.

\(^{98}\) *Id.* at 3.

and make competition in the industry in line with other industries. Boldrin and Levine recognize that reducing pharmaceutical profitability would potentially reduce drug development. However, they conclude that the immediate benefit of wider drug availability would exceed the long-term cost of having fewer new drugs.

To support their theory, Boldrin and Levine looked at recent legislation. They turned to the Bayh-Dole Act, which increased the intellectual property rights of universities and federally sponsored laboratories. They theorized that if patents do increase incentives to innovate, this legislation ought to have bettered the research of the universities that it benefitted. Boldrin and Levine, however, concluded that the quality of the research did not improve subsequent to the legislation and therefore levels innovation did not increase.

Finally, Boldrin and Levine looked to a British Medical Journal study in which readers nominated and then voted on pharmaceutical milestones. The

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100 Boldrin & Levine, supra note 142, at 262.

101 Boldrin & Levine, supra note 142, at 264.

102 Boldrin and Levine support their assertions by using a study by Hugh, Moore, and Snyder. This study actually has a contrary finding to that of Boldrin and Levine. Hughes et al conclude that “we would lose 3 dollars in benefits of innovation for every dollar we gain due to easier access.” They advocate keeping patent protection for pharmaceutical intellectual property. (James W. Hughes, Michael J. Moore, and Edward A. Snyder, “Napsterizing” Pharmaceuticals: Access, Innovation, and Consumer Welfare, National Bureau of Economic Research, 39 (October 2002).

winners included Penicillin, x rays, tissue culture, ether (anaesthetic),
chlorpromazine, public sanitation, germ theory, evidence based medicine,
vaccines, the pill, computers, oral rehydration therapy, DNA structure,
monoclonal antibody technology, smoking health risk.\textsuperscript{104} Because of 15 winners,
only two involve patents, Boldrin and Levine surmise that patents were not
necessary to develop some of the greatest advances in medical technology.\textsuperscript{105}

Boldrin and Levine are correct in their assertion that the costs of
intellectual property should be weighed against the benefits of innovation brought
on by intellectual property rights.\textsuperscript{106} They have, however, been accused of
severely misrepresenting the studies they cite to come up with this conclusion.
Richard Gilbert wrote a review piece of Boldrin and Levine’s meta-study on
pharmaceutical patents.\textsuperscript{107} He concluded that while the surveys cited by the two
scholars do take a critical view of the patent system, the overwhelming conclusion
of these studies was that patents are a piece of a broader strategy to protect
inventions.\textsuperscript{108}

\textsuperscript{104} The special issue of the BMJ in which this editorial piece appears lists the top 15
medical milestones and is available at www.bmj.com.

\textsuperscript{105} Boldin & Levine, \textit{supra} note 142, at 258–59.

\textsuperscript{106} Gilbert, Richard, A World Without Intellectual Property? Boldrin and Levine, Against
Intellectual Monopoly, Institute of Business and Economic Research, 4 (U.C. Berkeley,
February 1, 2010).

\textsuperscript{107} In 2008, Boldrin and Levine gathered 24 studies that examined patents and innovation.

\textsuperscript{108} Gilbert, \textit{supra} note 151, at 6–7.
“[B]y denying any positive role for intellectual property, Boldrin and Levine go further than the evidence can support without providing new evidence to justify their conclusions. The survey results reported . . . do not conclude that patents play no role to appropriate value of investment in R&D and thereby stimulate innovative efforts, but only that other mechanisms are often cited more frequently.”

Essentially, Gilbert states that Boldrin and Levine advocate limiting the role of patents in pharmaceutical innovation without “considering the potential hazards in their preferred world.”

3. The Advantages of the Monopoly Structure - Joseph Schumpeter

At the same time as Kenneth Arrow advocated competition for innovation, Joseph Schumpeter studied market concentration for the same purpose. Schumpeter argued that perfect competition is “inferior in internal, especially technological, efficiency.” Perfect competition is inferior because competitive firms waste opportunities and capital in trying to compete. This is why, according to Schumpeter, perfect competition is temporarily suspended whenever something new is introduced.

Before delving too deeply into Schumpeterian logic, we must first start by defining what it is Schumpeter actually meant by “competition.” Competition can

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109 Gilbert, supra note 151, at 7.
110 Gilbert, supra note 151, at 8.
112 Id. at 105.
be in the form of both price competition and competition by innovation. Competition by innovation, according to Schumpeter, hits “not at the margins of the profits and the outputs of the existing firms but at their foundations and their very lives.” That is, innovation can destroy existing firms that do not also innovate. This is commonly referred to as Schumpeter’s theory of “creative destruction.”

The temporary suspension of competition is justified by the difficulty many firms face in securing credit for research and development efforts. When firms must borrow to purchase real property, lenders are willing to lend because they will have a secured interest in that real property if the lender defaults. Conversely, when firms must undertake R&D activities, lenders realize that failure is likely, which limits any opportunity for collateralization: If the lender can only receive collateral in the product resultant of the R&D, they are unlikely to loan money because they realize that there is but a limited chance of the finished product’s being worth anything. Innovative firms, therefore, may not have sufficient access to credit to fund their innovations.

In response to these constraints, Schumpeter argued that monopoly competition provides protection “against temporary disorganization of the market” and secures space “for long-range planning.”

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113 Schumpeter, Joseph A., CAPITALISM, SOCIALISM AND DEMOCRACY 84 (1942).
114 Id., at 103.
argued that monopoly structures also offer “superior methods,” not available in competitive environments that enable monopolists to innovate because they don’t have to deal with “heavy capital requirements or lack of experience.”

Schumpeter’s contention is that the monopoly structure is well suited for innovation when there is significant risk involved.

While much of Schumpeter’s arguments focus on the suitability of monopolies in innovation, he argued that being a large firm is also sufficient to spur innovation. This is because the essential issues that Schumpeter is attempting to address are that of experience, financial resources, and capital requirements. Large firms have access to credit in that they have more collateralizable resources. Large firms may also have a greater cash reserve with which to finance R&D. Finally, large firms may simply have more resources at their disposal. Therefore, according to Schumpeter, both a monopoly market structure and a large firm size can allow for innovation.

While large firms also offer a conduit for development, Schumpeter considers monopolies a strategically important force for social progress. When firms are able to earn above normal profits, they have an incentive to enter a market and innovate. Therefore, according to Schumpeter, monopoly profits

\[\text{Id., at 89, 101.}\]
“might still prove to be the easiest and most effective way of collecting the means by which to finance additional investment.”\textsuperscript{116}

C. Patent Recalibration

1. Optimal Patent Coverage

“Traditionally, . . . patent breadth has been deemed the sole balancing element with the patent protection mechanism.”\textsuperscript{117} Breadth has been viewed as a better instrument than length to facilitate the best social outcome in regards to patents.\textsuperscript{118} Patent breadth refers to the scope of exclusivity afforded by a particular patent: The broader the scope, the more valuable the patent. At the outset, the tools available to the patent holder that can help determine patent breadth include compulsory licensing, experimental use, inventing around patents, and humanitarian-motivated social obligations.\textsuperscript{119} The judicial branch can further determine patent breadth through litigation.

Amir H. Khoury proposed recalibrating patent terms in a “case sensitive, differential manner.”\textsuperscript{120} He questioned whether the breadth of a patent by itself

\textsuperscript{116} Id. at 87.

\textsuperscript{117} Khoury, supra note 79, at 375.


\textsuperscript{119} Khoury, supra note 79, at 379–84.

\textsuperscript{120} Id.
would constitute a manner in which to achieve a social equilibrium of optimum patent protection. He proposed reaching optimum patent coverage by inducing the patentee to innovate, while not excluding either other innovators in the same field or consumers. Khoury attempts to find a system recalibration that accounts not only for the exclusionary interests of inventors, but the inclusionary social goals “for which purpose the patent concept was originally conceived.”

According to Khoury, a patent system that uses only patent breadth is no longer viable. Instead, Khoury advocates a balance between patent breadth and patent length for optimized patent coverage. This is because the incentive for innovation is contingent on the patent length, the patentee’s rights granted by law (i.e. patent breadth), and the commercial viability of the product. All three of these factors are interdependent: That is, patent length ought to vary based on patent breadth and patent length is contingent on the commercial viability of the product.

The relationship between patent length and commercial capacity, according to Khoury, ought to be inverse: The longer the patent length, the less commercial capacity associated with a particular product. Thus, calculating the commercial capacity of a product enables a society to formulate specific patent terms for distinct types of innovation. This implies that the broader the target

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121 Khoury, supra note 79, at 387.
122 Khoury, supra note 79, at 408.
audience for a product, the shorter the allowable patent length. Khoury points out that “[t]he model is especially relevant in the pharmaceutical sector, where the time between filing the patent application and receiving the patent registration is generally commercially worthless.”\textsuperscript{123} This commercially worthless period limits the commercial capacity of the product and, under Khoury’s model, permits a longer patent term.


William Nordhaus considered specific inputs in determining optimal patent life. Nordhaus stipulated that the formulation of an optimal patent term requires reaching equilibrium between the requisite incentives for innovation and the inefficiencies from patent monopoly rights.\textsuperscript{124}

To create his model, Nordhaus first considered what it took to actually invent. He, of course, observed that to make an invention, R&D costs must be incurred. He coined the term “invention possibility frontier” (“IPF”) which relates the output for innovation to expenditures on R&D. To simplify his analysis, Nordhaus used a simple invention possibility function with continuously diminishing marginal returns to inventive activity. Nordhaus’s IPF demonstrates that the more R&D time spent, the greater the cost savings, *ceteris paribus*.

\textsuperscript{123} Khoury, *supra* note 79, at 409.

Upon development of an innovation, under Nordhaus’s model, a firm has two options. It can either secure a patent and exclude other firms with now inferior goods or processes from competing, or it can license the patent to those other firms and secure royalty rents. In both of these scenarios, the innovative firm secures some monopoly power. However, this power does not enable the firm to charge a price above the cost associated with what would have been charged in the previous competitive market. As a result, the innovative good’s demand is not very elastic around the competitive price. The optimal price and quantity for the innovative product will be the same as the price and quantity of the product in the pre-invention equilibrium. The price will be lower if the invention reduces the long-run cost curve for the monopolist and reduces the marginal revenue curve to below the industry output in the previous competitive market.

Nordhaus noted that the longer the patent life afforded a firm, the greater the firm’s R&D expenditures. However, there is a balancing act to be performed here because patents involve some cost to society.
Figure 1 above demonstrates the conventional supply and demand diagram. The competitive price and quantity achieved by firms pre-patent is demonstrated by \( C_0 \) and \( X_0 \). The firm that secures a patent in this industry can then continue to charge the competitive price of \( C_0 \), but with a unit cost of only \( C_1 \). This cost reduction grants the firm a monopoly rent of \( C_0BHC_1 \). The price to society of this cost reduction from the time the invention is made public until the time the patent expires is the triangle BHA, plus the R&D costs of the inventor. The optimal patent life is achieved by balancing the temporary societal cost represented by the welfare triangle BHA and the R&D costs to the producer against the consumer and producer surplus created by the cost reduction of the innovation.

Through a complex algebraic analysis, Nordhaus determined that each additional year on a patent’s length produces less and less incremental cost reduction because later years’ monopoly rents are heavily discounted. At some point, the diminishing returns on patent length overpower any interest in stimulating cost reduction.

There are three important elements to be gleaned from Nordhaus’s model. First, the greater the elasticity of demand both before and after the innovation, the shorter the optimal patent length. This is because as price elasticity increases so does social welfare loss, which is captured in triangle BHA of Figure 1 above. Second, the easier it is for the innovator to achieve the cost reduction, which results in a steeper IPF and a larger equilibrium induced level of cost reduction, the shorter the socially optimal patent life. When an innovation compels large cost
reductions, society is less likely to defer its net welfare surplus for even more cost reductions. Finally, the optimal patent life is shorter where cost reduction effect of the innovation is small. Society is deferring the welfare gain while the patent is in place. If the cost reduction associated with the invention is small, the competitive price subsequent to the patent will not be much lower than the price during the patent. Society’s welfare gain subsequent to the patent will therefore be less.

Nordhaus concluded that inputs such as research and development cost and social value ought to be determinative in the patent life for a particular product. He noted that patent grants have a stimulus effect on investment. “[A]n optimal patent policy sees to it that the monopoly rent lure induces R[&]D investment just sufficient to equate the marginal social gain from further cost reduction with marginal social cost.”125 His work suggests that optimal patent life is extremely sensitive to the elasticity of demand, the discount rate on monopoly rents, and the importance of the particular invention. Depending on these inputs, Nordhaus opined that an optimal patent life should range from 1 to 34 years in length.126

In response to Nordhaus’s book, F.M. Scherer proposed a significantly more flexible variable patent system. He developed this system by cannibalizing and adapting the system proposed by Nordhaus. Unlike Nordhaus, Scherer


126 Id.
proposed an inflected IPF demonstrating initial increasing returns to research efforts that turn into diminishing returns.

Scherer departs slightly from Nordhaus in his analysis of patent investment inducement. While Nordhaus advocated the stimulus effect of patents, Scherer added to that effect what he termed the Lebensraum effect role of patents.\textsuperscript{127} Under this role of the patents, investors must be convinced that the patent will deter competitive imitation long enough to sufficiently compensate R&D outlays with discounted monopoly rents. Scherer argues that both the stimulus effect and the Lebensraum effect must be present to induce innovation.

Finally, Scherer suggested that patents should initially be given a relatively short term of protection, but with the option of receiving extensions. These extensions would be based on the ability of the inventor to prove that the invention deserved greater protection due to the research and development spend and social welfare benefits.\textsuperscript{128} He contends that uniform patent terms grant excessive payment in some cases. Essentially, Scherer advocated using the same


characteristics as Nordhaus to determine patent length but with the flexibility of allowing for governmental discretion of the overall length during the term of the actual patent coverage.
IV. Incentivizing the Development of Orphan Drugs: Patents or Alternatives

A. Orphan Drugs

The term “orphan drug” refers to those pharmaceuticals that treat very small patient populations known as rare diseases. Congress has defined a “rare disease or condition” as affecting fewer than 200,000 people or, if it affects more than 200,000 people, a drug a manufacturer would not reasonably expect to be able to recover the costs of research and development through sales in the United States.\(^{129}\) Currently, there are more than 6,000 of these types of disease that affect approximately 25 million people in the U.S. Some of these diseases affect fewer than 100 people.\(^{130}\) Prior to Congressional attention in 1983, these diseases were by-and-large neglected by pharmaceutical manufacturers due to the expected low potential for returns.

Due to their low profit prospects, orphan compounds have historically been discovered while researching the treatment of more prevalent diseases.


Accordingly, their use and treatment potential were discussed in publications without practical pursuit. In response to this, Congress enacted the Orphan Drug Act (“ODA”) in 1983 to provide incentives to drug manufacturers. This program has been generally touted as a success. Approximately 400 drugs and biologic products for rare diseases have been marketed under the program since 1983. This is a significant increase from the 10 such products that came to the market in the decade prior to the ODA’s enactment. However, these claims omit to account for simultaneously held patents by ODA program participants. They also don’t explain the effect of Hatch-Waxman that was enacted just one year subsequent to the ODA.

A report issued relatively soon after the ODA’s enactment found that:

“[T]he incentives offered by the Orphan Drug Act are not compelling enough to warrant the diversion of corporate resources towards discovery and development of products for the rare diseases.”

This is because the cost of regulatory approval for pharmaceutical companies pursuing treatments for these types of diseases still outweighs the expected

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revenues.\textsuperscript{134} Even with the ODA, pharmaceutical companies are still compelled by market forces to pursue drugs not targeted towards rare diseases.

While attempting to discern the effectiveness of the ODA, the National Commission on Orphan Diseases performed a study asking 35 pharmaceutical companies what compelled them most to engage in rare disease R&D. The respondents stated that the seven-year market exclusivity period induced them most to engage in R&D.\textsuperscript{135} Market exclusivity, in this case, is independent from, although functionally equivalent to, patent protection. During the market exclusivity period, competing firms can develop and/or imitate the drug; however, they are forbidden from bringing it to market until the exclusivity period ends. Accordingly, the market exclusivity period established under the ODA provides less protection than does a patent.

In his 2006 paper, Robert Rogoyski investigated the apparent triumph of the ODA and determined many of the successes attributed to the Act were more likely due to patent rights. He determined that 72 percent of drugs that fall under the act had patent rights that expire after the ODA market exclusivity period.\textsuperscript{136} The market exclusivity period had absolutely no effect on these drugs since their patents provided a much broader type of exclusivity. The orphan drugs also did


\textsuperscript{135} Nat’l Comm. on Orphan Diseases, Final Draft Report, 98, at 99 (Feb. 24, 1989).

not seem to affect the populations likely to be initially targeted by rational pharmaceutical companies. The overall average population of people affected by these diseases was 67,200. If the causal link were explained by market exclusivity alone, a rational firm would seek to direct its R&D efforts towards diseases affecting larger populations first.\footnote{137}{Id., at 4.}

Instead, Rogoyski suggests that stronger patent rights explain the increase in orphan drug development. He points to the 1980 Supreme Court decision holding that microorganisms are patentable.\footnote{138}{See generally, Diamond v. Chakrabarty, 447 U.S. 303 (1980).} He also acknowledges the pro-patent stance of the CAFC, which centralized the appellate court for patent cases.\footnote{139}{Rogoyski, supra note 181, at 4.} Finally, he points out that the Hatch-Waxman Act was enacted one year after the ODA and strengthened patent rights for pharmaceutical companies by granting five-year extensions.\footnote{140}{Rogoyski, supra note 181, at 4.} The five-year extension was intended to enable companies to regain time lost during the FDA approval process. Given that the majority of ODA program participants also have patents on their drugs, the market exclusivity period has been effectively rendered useless.

Bearing in mind that, as Rogoyski found, 72 percent of drugs that qualify for ODA marketing exclusivity also have patents providing much broader levels of protection, it can be concluded that the ODA does not, in fact, provide any

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\footnote{137}{Id., at 4.}
\footnote{138}{See generally, Diamond v. Chakrabarty, 447 U.S. 303 (1980).}
\footnote{139}{Rogoyski, supra note 181, at 4.}
\footnote{140}{Rogoyski, supra note 181, at 4.}
protection specific to drugs that treat rare diseases. Drug manufacturers have as much regulatory incentive to research and develop drugs that have larger end-user markets as they do rare diseases, thanks to provisions in the Hatch-Waxman Act. The larger potential profit of drugs that have the greater end-user markets, however, forces them, as rational market players, to pursue research for more ubiquitous diseases.

B. Why Patent Terms Should be Extended to Incentivize Orphan Drug Development

A differential system that provides incentives to firms to develop drugs for disenfranchised individuals is likely to produce a more efficient outcome. The most effective patent system equates marginal social cost to marginal social welfare. Thus, those patented innovations that produce a high ratio of deadweight loss to social welfare ought to have reduced patent terms. That is because for those products, marginal social cost meets marginal social benefits much sooner than patented innovations with a low rate of deadweight loss to innovations.¹⁴¹

The issue then becomes measuring the ratio of deadweight loss to welfare.

Figure 2 below looks at social welfare on a product with a patent and compares it to the social welfare on that same product without a patent.
Figure 2

$P_M = \text{Price with Patent}$

$P_C = \text{Price with Competition}$

$Q_M = \text{Quantity with Patent}$

$Q_C = \text{Quantity with Competition}$

$B = \text{Consumer surplus with patent}$

$H = \text{Producer surplus with patent}$

$B + H + A = \text{Consumer surplus without patent}$
The figure shows that the price of this product with a patent in place ($P_M$) is higher than its price without the patent ($P_C$). Furthermore, there is a much lower quantity of this product available in the market. The producer surplus earned under the patent is the rectangle labeled H, whereas the consumer surplus under the patent is the triangle labeled B. The deadweight loss in this scenario is represented by loss to the consumer and is contained in the triangle labeled A.

Deadweight loss as applied to patents on drugs that treat rare diseases is somewhat misleading. That is because deadweight loss defines a situation in which people are being priced out of a specific market. Pharmaceutical patents on orphan drugs create deadweight loss for a product that otherwise would not exist. This is true in the case of drugs that treat rare diseases because, as previously noted, prior to the enactment of the ODA and the Hatch-Waxman amendments, only ten drugs of this category existed: Subsequent to these two pieces of legislation, more than 400 orphan drugs existed. Thus, when considering deadweight loss in the context of orphan drugs knowing that these drugs would not exist if not for development incentives, it is especially important to weigh both sources of consumer surplus: The sale of branded drugs under the patent and the sale of drugs off patents.\(^{142}\)

Hughes, Moore, and Snyder performed a study in which they determined that welfare losses due to patents causing drug prices to exceed marginal costs

\(^{142}\) Hughes, Moore, & Snyder, supra note 81, at 21.
ought to be netted out by consumer surpluses derived from the drug.\textsuperscript{143} This is because they considered surpluses to include the more intangible aspect of the drug’s development itself, although much of this value is not achieved until the drug goes off patent and is available through generic brands. Essentially, a longer timeframe must be considered to truly measure welfare losses against consumer surpluses.

The static gains in consumer surplus from removing patents are substantial, but are dwarfed by the dynamic losses in consumer surpluses that come from the removal of the innovation incentive.\textsuperscript{144} However, patents that protect a longer period than necessary to induce development are fundamentally inefficient. This is because for some period of time, these patents provide market exclusivity without providing any social benefit. Thus, they grant unnecessary monopoly rents and induce rent-seeking behavior.

Rent-seeking behavior is a negative externality of patents. It helps to deter competition by increasing regulation and barriers to entry for potential competitors. However, in the case of orphan drugs, monopoly rents are small due to the limited end-users. While extended patent terms are intended to increase these monopoly rents, they are unlikely to increase profits to a magnitude that would induce rent-seeking behavior. These drugs treat populations of fewer than 200,000 people. Rational firms will not find it cost effective to spend time and

\textsuperscript{143} Id.

\textsuperscript{144} Hughes, Moore, & Snyder, supra note 81, at 28.
resources lobbying to exclude potential competitors for markets of this size. Even with extended patent terms, pharmaceutical companies can still expect to reap profits much smaller than that necessary to warrant regulatory manipulation.

While rent-seeking behavior may not be a major concern in this context, regulated access to developments that can be used to compel future developments might be. Arrow recognized that inventive activity is both an output and an input to new development. Patents can potentially stifle this future development. In the case of orphan drugs, which are currently inadequately addressed, patents may be used to increase the initial inventive activity that can eventually become an input in future development. It is unfortunate and unavoidable that by doing so, future inventive activity based on existent innovation may be delayed. However, without the incentive to create the seed invention, future outputs incorporating that invention will be delayed indefinitely.

C. Alternatives to Patents

Since the free-rider effect is inherent in innovation, any government that seeks to promote improvement must develop a mechanism to overcome this problem. There are generally three distinct categories of systems that can alleviate this issue: (1) legal solutions that use coercive power to create and enforce, rights and redistribute resources; (2) anarchic or quasi-legal systems that depend on a
sense of moral duty; and (3) business-based mechanisms that enable the firm to appropriate returns from their research and development investments.145

1. Legal Solutions

Legal solutions offer the advantage of bright line rules and tangible enforcement. They include such strategies as monopoly rights, government rewards, government subsidies, and trade secrets. As it stands, virtually all nations apply some kind of legal solution to facilitate innovative incentives.

Monopoly rights are the first and most often adhered to legal solution to the free-rider problem. This category includes utility patents, design patents, plant patents, and copyrights. A monopoly right is granted for a limited time and knowledge is transferred into the public domain at its expiration. Monopoly rights enable the innovator to control the future of the innovation during the patent/copyright’s term, which allows for a better managed innovative exploitation. Furthermore, knowledge is more readily disbursed since public disclosure is required as a condition for patent protection. Such disbursement aids eventual competition at the expiration of the monopoly right.146

The government can provide direct rewards to inventors in order to spur innovation. This method can, theoretically, reduce deadweight loss that is


146 Johnson, supra note 190, at 272.
associated with the limited time monopoly rights patent system. However, there are logistical and administrative difficulties with determining the adequate reward for particular inventive endeavors. This problem is especially pronounced when there are improvements to existing inventions. Finally, this method possesses discretionary issues with determining what qualifies as an innovation worth rewarding.\footnote{Johnson, supra note 190, at 273–74.}

In a similar manner to direct government rewards, the government may also offer subsidies. These subsidies may be offered prior to the commencement of the research. This allows firms to undertake research and development projects that they would otherwise be unable to finance. It also allocates risk to the government as opposed to the innovator. However, it is immensely more difficult to estimate innovation’s value ex ante than it is ex post, which would make any valuation attempts potentially inaccurate.\footnote{Johnson, supra note 190, at 274–75.} Inaccurate subsidy valuation would misdirect innovative projects.

The final major legal strategy that may promote innovation is a system of trade secrets. Trade secrets involve ideas, know-how, or information that may be protected by a contract regime with special remedies. They protect an expansive set of innovations; however, they allow other firms to legally appropriate innovations simply by duplication of work already performed. This not only provides disincentive for original innovation, but is also a waste of resources. In
addition, the concept of trade secrets is incompatible with allowing innovative knowledge to pass into the public domain. Trade secrets have no expiration or method for transference so they provide either perpetual monopoly pricing or duplicative R&D spending.¹⁴⁹

2. Quasi-Legal Systems

Quasi-legal schemes generally rely on innovators’ internal motivators and, in some cases, the drive and commitment of external organizations. The methods that fall under the Quasi-Legal category include non-profit support, pride/internal motivation of the innovator, “copyleft”, and shareware. These methods all rely heavily upon the ethical behavior of product users. This, of course, leads to the potential for moral hazard.

Non-profits are the organizational standalone in this group. They may allow for the appropriation of research and development expenditures and are especially compatible when the social value exceeds the business value of an invention. This method relies on tax-exempt non-profit corporations to supply funding for research and development. The obvious issue is that these firms rely entirely on the voluntary remuneration of funds from the general public. They are especially susceptible to economic downswings that can cut-off funding. These

¹⁴⁹ Johnson, supra note 190, at 276–77.
rewards do have the benefit of flexibility in funding timing since they can be offered either ex post or ex ante, depending on the organization’s structure.\footnote{Johnson, \textit{supra} note 190, at 276.}

The internal motivation of the inventor comes heavily into play under the remaining structures under this category. Pride/internal motivation driven by the internal desire to invent for self-actualization is a very powerful stimulus for many people.\footnote{Johnson, \textit{supra} note 190, at 276–77.} Copyleft/patleft, which is used by some computer hackers, combines contractual licensing and anarchic values to create large programs with numerous different authors. The primary hacker retains the program’s copyright and distributes the program to others on the condition that if other programmers constructively add to the code, they make that addition available to others on the same terms as the original license.\footnote{Johnson, \textit{supra} note 190, at 277.} Finally, shareware operates on a similar moral basis as copyleft. Programmers release their programs for free in the public domain and request users voluntarily pay a license fee.\footnote{Johnson, \textit{supra} note 190, at 277.}

3. **Business-Based Mechanisms**

In addition to separately considered mechanisms to stimulate innovation, there are some mechanisms that are intrinsic to the business structure itself. Businesses that are “first-movers” benefit from the lag that rival firms will
experience in trying to duplicate a profitable new technology. It takes time to hire
or retrain personnel, to retool or purchase new equipment, and to change the
firm’s marketing focus.\textsuperscript{154} Similarly, some firms are able to appropriate returns
from innovations by moving along the learning curve quickly, again leaving their
competitors behind.\textsuperscript{155} These methods both rely on the adaptability of a particular
firm, as well as the inadaptability of that firm’s competitors.

Other business strategies that can facilitate a firm in actualizing
innovations can be built into the firm’s business plan itself. These include: (1)
superior sales and service efforts of a firm; (2) the manufacturing capacity of that
firm; (3) the firm’s increasing returns; and (4) proprietary architecture.\textsuperscript{156}
However, all of these methods for appropriation rely on one firm being
significantly more efficient than its competitors. In a perfectly competitive
market, this is unlikely to be the case.

4. Public Funding

Public funding for pharmaceutical development makes sense when the
social returns to the basic research exceed the private returns that can be expected.
In the U.S., federally funded programs have greatly shaped pharmaceutical

\textsuperscript{154} Johnson, supra note 190, at 279–79.

\textsuperscript{155} Johnson, supra note 190, at 279.

\textsuperscript{156} Johnson, supra note 190, at 280 (If the innovative product requires other products to
work effectively, the controlling architecture facilitates the appropriation of research and
development efforts.).
innovation. Approximately 41.4 percent of drug manufacturer R&D projects use public research findings. Given the presence of public funding in the pharmaceutical industry, it seems reasonable that public funding is a manner in which to positively affect pharmaceutical innovation.

“Push” programs in the pharmaceutical industry are intended to subsidize the cost associated with drug discovery and therefore push the development of new drugs. These programs include the public subsidy of basic research and the public subsidy of phase III clinical trials. Basic clinical research is the research that takes place at the very beginning of a new drug’s development. The exceedingly high failure rate associated with this stage of development is the reasoning for targeting it for public funds. “One option is public subsidy of large-scale, not-for-profit consortia that conduct the basic research necessary to identify and validate drug targets in humans.” This would lead to a database accessible to all pharmaceutical companies that would form the basis of much of their future research.

Another manner in which to subsidize drug development is publicly funding phase III clinical trials. Phase III trials tend to be the most expensive single aspect of drug development. Public funding of these trials is logical


because: (1) the government already spends money on phase III trials when determining whether or not to approve a drug; (2) the cost of capital faced by the government is likely to be less than the cost of capital faced in private industry; (3) if phase III trials are publicly funded, regulators are less likely to impose additional restrictions on their conduct; and (4) the information on safety and efficacy of the new drugs is more likely to be credible and clinically useful. In other words, the government is already heavily present in phase III trials so the infrastructure is conducive to additional funding.

To determine the viability of public funding on pharmaceutical research and development, Margaret Blume-Kohout analyzed the effects of spending by the U.S. National Institutes of Health ("NIH"), which provides grants for research focused on specific diseases. She found that there is a significant positive, lagged effect of NIH funding on specific disease targeted drugs that enter the first phase of clinical trials: An increase in NIH funding of ten percent results in a 4.5 percent increase in drugs going to initial clinical trials. Blume-Kohout also found that the ten percent increase in this targeted funding increases the number of drugs going to phase III trials by approximately two percent. There is a twelve-year lag associated with these increases. However, Blume-Kohout has found that public funding does effectively increase pharmaceutical R&D.

159 Id.
161 Id.
There are many concerns with government funded innovation schemes. The first stems from the possibility of budget cuts that could deter innovation. Pharmaceutical companies would need to trust that the government would continue to fund research and development. In times of national economic contraction, this could be a difficult leap of faith. In addition, there is the possibility of waste. If the pharmaceutical companies themselves are not funding research and development they may have incentive to pursue research paths with little likelihood of success. Even with these potential negative influences, public funding is a viable manner in which to induce research and development.

D. Why Patents are the Least Worst Option to Incentivize R&D for Rare Diseases

This paper has explored the many pitfalls of the patent regime. The nature of drug development as a public good and the requirement to protect and monetize that knowledge leaves no viable alternatives. For example, if the government were to attempt to resolve the public goods problem by developing the good, enabling a shared database of information, or providing subsidies for private development, it would have to act as an omniscient being to prioritize the treatment of ailments and determine adequate R&D expenditures. Furthermore, the government is susceptible to funding issues because the money necessary to run these types of programs would have to come from sometimes-whimsical taxpayers. Enacting a competitive market place for drugs would retard innovation because these products are cheaper to copy than develop. Prices can certainly be
regulated as they are in other countries, but that speaks to a structural industry consideration and not the relevance of patents in promoting innovation.

Risks incurred by drug companies in attempting to discover drugs for limited populations are even greater than those faced to discover “normal” drugs. As Schumpeter argued, monopoly competition provides reassurance against the disorganization of the market at these initial stages and allows for long-term planning.162 He further contended that the patent pricing structure allows for innovation.163 Drug companies considering this type of risky endeavor need the reassurances Schumpeter references.

The patent induced monopolistic pricing certainly has some negative externalities, but there are no viable structural alternatives. Pricing drugs targeted towards rare diseases at their marginal cost would not cover the associated R&D costs. The current patent term of 20 years, which generally establishes an eleven-year effective patent term, has thus far proved inadequate to sufficiently incentivize development of orphan drugs. The assistance gained from the ODA is tenuous at best because its enactment closely aligns with strengthening patent rights for pharmaceuticals under Hatch-Waxman. While the time period in which both pieces of legislation were effective offered more drugs to treat rare diseases,

162 Schumpeter, Joseph A., CAPITALISM, SOCIALISM AND DEMOCRACY, 103 (1942).
163 Id., at 89 & 101.
many more of these diseases are still commercially unviable for drug developers. Since there appears to be no better way to incentivize invention than patents, prolonging patent rights for drugs targeted towards rare diseases appears to be the best solution.
V. Conclusion

The pharmaceutical industry is heavily criticized. The beneficial nature of the products it develops seems to induce an expectation of altruistic motives in its operation. Despite this, pharmaceutical companies are rational, profit-seeking firms. They rationally choose not to engage in the production of goods they do not foresee being profitable. They use legal means to cover initial costs of production and consider these means when determining what products to develop. While pharmaceutical companies have abnormally high profit margins when compared to other firms of similar size and maturity, they justify these profits based on the risk of their business.

Patents are almost as controversial as the pharmaceutical industry. Despite their controversy, scholars have yet to propose a better incentive for innovation, especially in the drug development world. Patents offer the patent holder the opportunity to earn monopoly profits for a set period of time in order to reward their inventiveness. This is a requirement for rational profit-seeking firms in the business of invention, as is the case in the pharmaceutical industry.

Extended patent terms offer the best way to incentive pharmaceutical companies to engage in development of drugs that otherwise would not be
commercially viable. These drugs are targeted towards the often disenfranchised group suffering from a rare disease. By extending patent terms, drug companies can expect to earn back the money initially sunk into development. The length of the extension is beyond the scope of this paper, but would be an interesting area of future analysis.
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