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*JAMA*. 2005;294(16):2075-2082 (doi:10.1001/jama.294.16.2075)

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# The Patents-Based Pharmaceutical Development Process

## Rationale, Problems, and Potential Reforms

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**T**HE PHARMACEUTICAL INDUSTRY faces substantial criticism from many directions.<sup>1-3</sup> Some critics focus on barriers to access to drugs in both developed and developing countries. Others focus on continued high profits masking growing difficulties in developing new pharmaceuticals and the lack of incentives for products addressing diseases in developing countries. Further criticisms focus on the advertising and marketing budgets. Underlying these criticisms are fundamental questions about the value of the patent-based drug development system: Is the patent system an effective way to ensure the development of new, medically useful pharmaceuticals? What alternatives to the patent system exist? What are their advantages and disadvantages?

### Current Criticisms and Concerns

While the high costs and cost increases of drugs promote drug development, they also significantly restrict access.<sup>4</sup> From 1992 to 2002, retail prices for prescription pharmaceuticals in the United States increased 7.3% annually, greatly exceeding the 2.5% annual inflation rate.<sup>5</sup> In 2002, drug costs accounted for 10.5% of all health expenditures, nearly twice the level of 5.8% a decade earlier (FIGURE 1).<sup>6</sup> Many of these pharmaceutical expenditures

The pharmaceutical industry is facing substantial criticism from many directions, including financial barriers to access to drugs in both developed and developing countries, high profits, spending on advertising and marketing, and other issues. Underlying these criticisms are fundamental questions about the value of the current patent-based drug development system. Six major problems with the patent system are (1) recovery of research costs by patent monopoly reduces access to drugs; (2) market demand rather than health needs determines research priorities; (3) resources between research and marketing are misallocated; (4) the market for drugs has inherent market failures; (5) overall investment in drug research and development is too low, compared with profits; and (6) the existing system discriminates against US patients. Potential solutions fall into 3 categories: change in drug pricing through either price controls or tiered pricing; change in drug industry structure through a "buy-out" pricing system or with the public sector acting as exclusive research funder; and change in development incentives through a disease burden incentive system, orphan drug approaches, or requiring new drugs to demonstrate improvement over existing products prior to US Food and Drug Administration approval. We recommend 4 complementary reforms: (1) having no requirement to test new drug products against existing products prior to approval but requiring rigorous comparative postapproval testing; (2) international tiered pricing and systematic safeguards to prevent flow-back; (3) increased government-funded research and buy-out for select conditions; and (4) targeted experiments using other approaches for health conditions in which there has been little progress and innovation over the last few decades.

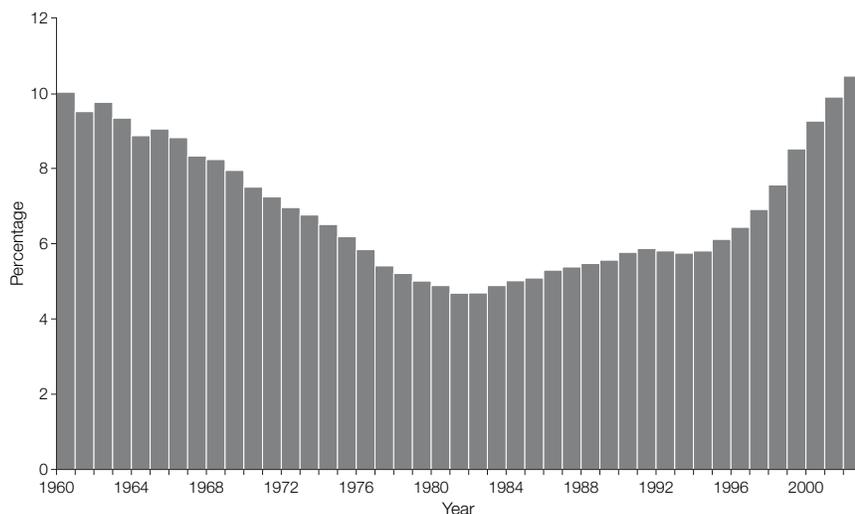
*JAMA.* 2005;294:2075-2082

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improve quality of life and save money by substituting for more expensive interventions.<sup>7-9</sup> However, US residents pay the high drug prices or copayments, while insurers and the government realize the savings. Simultaneously, drug costs limit access to

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**Figure 1.** Drug Costs as a Share of Overall Health Care Costs, 1960-2002

Data from Centers for Medicare and Medicaid Services.<sup>6</sup>

effective drugs in developing countries. While many factors, including poor infrastructure and lack of trained personnel, contribute to access problems, there has been global criticism of the drug industry's use of patents in developing nations.<sup>2</sup>

These trends, combined with the withdrawal of medications because of adverse effects, efforts to thwart the dissemination of negative research results, and conflicts of interest with academic researchers, have generated considerable worldwide public hostility toward the pharmaceutical industry and pressure for political responses, including calls for drug reimportation or price controls, tighter regulation, and international agreements permitting the override of drug patents.<sup>1-3,10</sup>

### The Rationale for Patents

There are 2 distinct rationales that might be given for patents.<sup>11</sup> An intrinsic rationale justifies patents as recognition of the inventor's creativity. This view has been important in continental European law. A second rationale is more instrumental; patents are a means to enhance innovation, the ultimate goal. Consequently, a patent is a right to exclude others; it is a right to a tem-

porary monopoly, permitting a higher price to be charged for the product, which in turn is supposed to stimulate innovation. This instrumental view is embedded in the US Constitution, which gives Congress the power "[t]o promote the progress of science and useful arts, by securing for limited times to authors and inventors the exclusive right to their respective writings and discoveries."<sup>12</sup>

To obtain a patent, an invention must be (1) novel—meaning that it has not been published more than a year before the patent application; (2) not obvious; (3) useful; and (4) adequately disclosed in the patent application to enable a scientist to practice the invention.<sup>13</sup> These requirements ensure innovation by precluding patents for something already invented. While harder to enforce than product patents, patents may be granted for methods of using an existing product for a new purpose.

### Patents in the Drug Development Process

The development of a drug is an enormously expensive process because of the high attrition rate of potential products as they proceed through laboratory, animal, and various human trials, as well

as the high costs of trials needed for regulatory approval. Only 21% of drugs that begin human testing are finally approved.<sup>5,14,15</sup> While contentious, the published average cost of drug development is approximately \$800 million, with half being actual outlays and the remainder being cost of invested capital.<sup>14</sup> In some cases, significant portions of these costs, particularly those incurred early in the development process, are covered through government-supported research.

The pharmaceutical industry recovers its expenses through charging a high price for the drug, typically based on exclusivity rights under a patent. When the patent expires, the price normally decreases through competition with generic drugs.<sup>15</sup> Consequently, use of a new drug by the first generation of patients covers the costs of the research. For a blockbuster drug, each month of exclusivity could be worth \$100 million or more. Importantly, the actual economic value depends on the value of the product to patients as well as on the presence of competitive products. In addition, marketing can lead to some degree of market power and higher prices, even in the absence of patents—this is the impact of brand names on generics.

The effective length of the patent monopoly is not the same as the formal 20 years from the date of application stated in patent law. To preempt competitors, companies must apply for patents early in the development process, while marketing exclusivity occurs only after trials lead to regulatory approval. Congress has adopted a number of patent law changes to compensate for the time lost during clinical trials and the regulatory approval process. The Hatch-Waxman Act of 1984 grants pharmaceutical companies additional exclusivity, increasing the effective exclusivity period from 8.2 to 11.2 years.<sup>5,16</sup> The act also created incentives for generic firms to enter the market immediately after patent expiration, to lower drug prices through competition. Legislation in the late 1990s, aimed at speeding up the regulatory process, increased effective ex-

clusivity to nearly 15.4 years.<sup>5,17</sup> In addition, under the 1983 Orphan Drug Act,<sup>18,19</sup> a shorter additional period of exclusivity may be given to a firm for use of a known product to treat an orphan disease, and amendments to the Food, Drug, and Cosmetic Act<sup>20</sup> provide for 6 months of additional exclusivity for certain drugs if extra testing is performed for pediatric applications. Finally, for many biotechnology products, there is currently no abbreviated process to approve a generic substitute. Unless current discussions to develop such a process succeed, the effective exclusivity period for these products may be far longer than the lifetime of the patent.<sup>21</sup>

### Changing Trends in Pharmaceutical Development

According to theorists, drug development is the paradigm of patents spurring innovation. However, at least since the mid 1990s, there has been a decrease in the number of fundamentally new products, ie, "new molecular entities."<sup>22,23</sup> The reasons for this are unclear. However, one group suggests that the decrease results from a greater emphasis on developing products for chronic and complex indications; the growing size of clinical trials; difficulties recruiting and retaining patients; increased regulatory and political pressures, especially in the United States, where the Food and Drug Administration (FDA) is requiring more extensive safety data sets for new drug applications; increasing clinical development costs; and poor returns on expensive discovery technologies.<sup>24,25</sup>

The search for "blockbusters" may contribute by focusing attention on a relatively small number of clinical goals that may prove increasingly elusive. Yet this focus is logical, because the industry's profits are dominated by profits on a small number of drugs.

Related to this decrease in the number of new products is the increase in research and development costs per new drug. Research, and especially clinical trials, is becoming more expen-

sive, requiring greater returns on the drugs that are approved, driving their costs up. The total pharmaceutical industry research cost per new molecular entity approved is increasing 10.3% per year, excluding funding by the National Institutes of Health and by biotechnology companies, nearly equivalent to the 10.4% annual nominal growth of health care expenditures.<sup>6,26</sup>

There is also a change in the organization of drug research.<sup>27</sup> Previously, the pharmaceutical industry emphasized internal research that searched libraries of compounds for those that would be effective against important biological targets. Today, biotechnology companies conduct much preliminary research, including early clinical trials, licensing their candidate compounds to the major pharmaceutical companies capable of managing large-scale trials and marketing. The major companies, in turn, frequently subcontract out the clinical trials portion of development to specialist clinical research organizations. Frequently, the biotechnology companies have licensed their technology from university researchers, sharing the monetary success of any resulting products. This process accounts for about 30% of new product approvals, while the number of traditional small-molecule drugs approved is declining rapidly.<sup>25</sup>

### Problems With the Patent-Based System

There are at least 6 distinct problems with the current pharmaceutical development system. First, recovering research costs through a patent monopoly intensifies the tension between research goals and accessibility goals. The more spent on research, the higher the drug price required to recover those costs—yet higher prices limit access to the innovative product.<sup>4</sup>

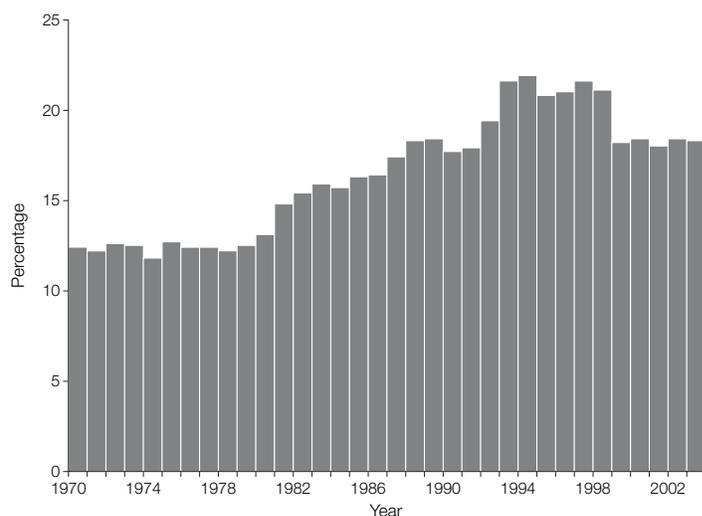
Second, potential level of demand influences research priorities. The system is ill-adapted to develop products of great social need with relatively small economic markets. The pharmaceutical companies invest very little in research on diseases endemic to devel-

oping nations, because there is no commercial market from which they can recover research and development costs.<sup>2</sup> Similarly, they have little incentive to invest in pediatric diseases, preventive interventions, vaccines, and relatively rare diseases that occur in developed countries. Consequently, pediatric exclusivity and the Orphan Drug Act<sup>18,19</sup> have been enacted. Similarly, to develop biodefense products under the Project BioShield Act of 2004,<sup>28</sup> the US government is being forced to use new procurement mechanisms.

Third, a misallocation of resources exists between research and marketing.<sup>5,9,24,29</sup> According to pharmaceutical industry publications, promotional costs, including the value of samples, amounts to about 60% of the research expenses. Other statistics suggest that for the 10 largest pharmaceutical companies, 13.7% of revenue is devoted to research and development, while 34.4% goes to marketing, general, and administrative costs, 29.4% to product manufacturing costs, and 23.6% to pretax profits.<sup>4,5</sup> While administration, manufacturing, marketing, and profits are necessary, the proportions seem excessive. The firms allocate greater resources to marketing because it yields greater returns than research on new drugs. Only a major change in the way drugs are marketed could change this balance between research and marketing.

Fourth, even without the patent monopolies, payment for drugs is not an ideal market. Today, about 67% of expenditures for drugs in the United States are covered by private insurance and the government.<sup>30,31</sup> Third-party payments distort market incentives and preclude traditional economic analyses.

Fifth, the overall level of investment in the development process actually seems low, given the profits. The financial return from pharmaceutical research has followed an inverted U-shaped curve.<sup>9</sup> In the 1970s, the percentage of sales spent on research was approximately 12.5%. It peaked at 22%

**Figure 2.** Pharmaceutical Industry: Percentage of Sales Spent on Research, 1970-2003

Data from *Pharmaceutical Industry Profile 2005*.<sup>9</sup>

in 1994 and has decreased to about 18% of sales (FIGURE 2).<sup>4,22,29</sup>

Finally, the existing system discriminates against US patients, employers, and taxpayers who pay for health care. With fewer price restrictions, US health care payers experience high drug prices, contributing 62% of all pharmaceutical profits.<sup>32</sup> Conversely, because of significant pharmaceutical price controls, European nations contribute a smaller proportion of profits and research investment. Consequently, many outside the United States—including well-off individuals in developed countries—are free riders on the research innovations derived from America's high drug prices.

### Potential Solutions

If the patent system for drugs is less than perfect, what alternatives might do better? The proposed alternatives can be categorized into 3 distinct types: those that (1) affect drug prices, (2) change the industry structure, and (3) alter the industry's incentives (TABLE).

**Price Controls.** One alternative is price controls in the United States. Large health maintenance organizations and pharmacy benefit managers, which cover approximately 67% of US patients, are bargaining down prices.<sup>30,31</sup> Although price bargaining is prohib-

ited under the new Medicare pharmaceutical benefit,<sup>33</sup> it seems likely that taxpayers will insist on some form of price reductions, if not outright control.

Adding price controls for drugs in the United States to price controls for those in other developed countries will not affect the supply of current drugs, because the marginal cost of production is negligible. However, over the long term, the use of price controls will decrease profits and the level of research, diminishing the future supply of new drugs. But because the real impact would only be felt a decade or more into the future, such approaches may have short-term political appeal.<sup>34</sup> Only by combining price controls in the United States with loosening of price controls in Europe to maintain the industry's profits would it be possible to maintain the same level of research and development. Since every developed country is facing health care cost pressures, this seems unlikely.

**Tiered Pricing.** Tiered pricing—ie, having higher drug prices in developed countries and lower prices in developing countries—is efficient, equitable, and enhances utility.<sup>35</sup> The entire African drug market contributes approximately 0.4% of the sales of the US pharmaceutical industry.<sup>9</sup> Having prices

near marginal cost in these markets would have little impact on drug development incentives. Although poor infrastructure also affects access to drugs for these nations, such tiered pricing would provide more equitable access to drugs.

Tiered pricing might be achieved by arrangements to compel the surrender of patent rights in developing countries.<sup>10,36</sup> More likely it will be based on the threat of compulsory licensing to a generic firm, which would be authorized to produce the product despite the original firm's patent. The 2001 Doha and 2003 Cancun declarations by the World Trade Organization created the legal arrangements for such licensing.<sup>10</sup> Importantly, no generic manufacturer is likely to be willing to produce the product unless it could cover its start-up and production costs. Would the poor nations be able to pay for those costs without some form of subsidy?

Tiered pricing does little to create incentives for the pharmaceutical industry to conduct research on the so-called diseases of the poor. Large price differentials create incentives for the reverse flow of products from developing to developed countries. One incident of smuggling \$18 million worth of antiretroviral agents from Senegal into Europe has been reported.<sup>37</sup> Radiolabeling, differences in color and shape, and other new techniques could minimize such smuggling. Furthermore, global communications will generate publicity about the marginal cost of drug production and cases of wealthy people in poor nations obtaining products much more cheaply than poor people in wealthy nations, creating political pressures in developed nations for lower prices.

Despite these problems, the positive aspects of tiered pricing almost certainly outweigh the negatives.<sup>35</sup> In addition, a global Hatch-Waxman-type compromise might provide increased returns to the pharmaceutical industry in developed countries in return for a greater role for the generic firms in developing countries.

**“Buy-out” Pricing System.** A buy-out pricing system gives a cash bonus of public money to the pharmaceutical firm for the estimated profits from monopoly prices. The pharmaceutical firm would then make the drugs available to everyone in need at the marginal cost of production. Apparently, the only case in which this approach was used was the purchase of the daguerreotype patent in 1839.<sup>38</sup> However, governments have used prizes as incentives for innovation in high-priority areas, such as a method to determine longitude.<sup>39</sup> The appeal of the buy-out is that it would spread the cost of drug development across all of society and make pharmaceutical products available to patients at the lowest possible price, while giving industry substantial research incentives.

Certainly, there will be significant debates on which products should be in-

cluded, the formula for pricing, and whether this should be voluntary with the patent holder. But the major limitation is whether there can be a good mechanism to estimate the patent’s value. There have been proposals for various auction structures.<sup>40</sup> Estimates of the likely return for a product are also possible, since boards of the major pharmaceutical companies make such estimates when they commit research and development funds to particular potential agents. But it is hard to estimate what competing products might emerge within the expected lifetime of the patent and how use of this product will compare with that of other available products. Furthermore, buy-out pricing would require political will: would governments be willing to provide buy-out funding?<sup>40</sup>

**The Public Sector as Research Funder.** Another alternative entails that

the government purchase the pharmaceutical products and explicitly cover the necessary research and development costs. This can be a “push” arrangement, in which the government directly supports the research costs in developing new products, or a “market-pull” arrangement, in which the government can promise to purchase and pay a markup on finished products at a level designed to cover and encourage research by pharmaceutical companies.

Drugs of great social value can be developed and procured, despite the absence of commercial markets and incentives. “Pull” arrangements also reduce costly drug promotional activities. Sponsored-research approaches are used in the defense industry for purchase of major weapon systems. Arguably, the “push” arrangements have long been used by the National Insti-

**Table.** Strength and Limitations of 7 Proposals to Reform the Drug Patent System

Type of Reform	Proposal	Strengths	Limitations
Change drug prices	Price controls	Make drugs more accessible by lowering prices Politically popular	Reduce drug companies’ incentive for research
	Tiered pricing	Makes drugs more accessible in developing countries Improves worldwide utility No compromise on research for drugs aimed at developed countries	Reduces drug companies’ incentives for research on diseases of the poor Reverses importation of drugs from developing back to developed countries Adverse publicity in developed countries about real cost of drug production
Change industry structure	Buy-out pricing system	Makes drugs accessible at marginal costs of production without decreasing research incentives	Difficulties determining which drugs should be bought out Difficulties determining a fair price for bought-out drugs Political will required to pay up front for drugs
	Public sector as research funder	Creates an incentive to develop drugs for which there is no market—eg, bioterrorism drugs Sector has experience with the system in defense and bioterrorism drugs	Few decision makers, leading to overlooking of important new drugs Inefficient drug development process from guaranteed returns Resistance by pharmaceutical industry due to low returns
Change development incentives	Disease burden incentive system	Returns depend on actual impact on global health Shifts focus away from trivial but high-market-demand interventions Allows pharmaceutical industry flexibility in how to address diseases	Difficulties in determining the actual effect on global health Requires restructuring of the entire health care sector Could compromise research in rare diseases with small impact on global health
	Orphan drug approaches	Encourage research on new categories of drugs, possibly including those relevant to developing nations Tax break version can decentralize decision making compared with public-sector procurement	Cost per patient may still be high Developing-nation versions probably require very substantial tax incentive
	Requiring products to demonstrate improvement	Shifts focus to drugs that make a significant impact compared with currently available interventions	Increases cost of drugs by substantially increasing cost of clinical research and reducing competition among drugs Not all similar drugs are really similar in effectiveness and adverse effects; would reduce variety of drugs

**Box. Recommendations for the Future****Requiring New Products to Demonstrate Improvement Compared With Existing Treatments**

Should not be required as part of US Food and Drug Administration approval process because it will substantially increase drug prices and limit the number of drugs with different adverse-effect profiles

Should be required postapproval—a company should perform at least 1 high-quality comparative research study with a comparator drug in the same class

**Tiered Pricing**

Should be required at marginal production cost for the poorest developing countries, with strict safeguards against smuggling

**Targeted Public-Sector Funding With Buy-out**

Should be used for products with limited markets, such as vaccines, new tuberculosis treatments, bioterrorism drugs, and drugs for diseases endemic to developing countries

**Limited Experiments With Other Approaches**

Should be tried for 1 area, such as water-borne diarrheal diseases or development of new tuberculosis drug

tutes of Health when it develops a drug or vaccine and then licenses it to a pharmaceutical company to conduct some of the final clinical tests and handle the regulatory approval process. Versions of this approach are already being used for bioterrorism products.<sup>28</sup> Push arrangements are also being used by foundation-supported Public Private Partnerships such as the International Aids Vaccine Initiative to develop drugs for specific diseases of concern to developing nations. Market-pull arrangements have been proposed under which funders, such as the World Bank, would promise to pay for vaccines meeting the needs of developing countries.<sup>41,42</sup>

The major limitations parallel those encountered in the defense industry. Fewer independent decision-making centers may lead to the ignoring of new research and product directions and to the politicization of decisions. Without market constraints this may also foster inefficient research and development. Finally, the pharmaceutical industry is likely to be resistant. By one measure, returns on assets in the defense sector are 3.3%, substantially lower than the 10.3% in the pharmaceutical industry.<sup>43</sup> Indeed, at least part

of the problem in vaccine development is the low return on investment for the pharmaceutical industry compared with that for other potential investments. Nevertheless, development of bioterrorism-related drugs and antibiotics to overcome drug resistance is likely to follow this approach.

**Disease Burden Incentive System.** An approach that has not been widely explored is for the government to pay for drug development, with the price reflecting the social value of its contribution to reducing global disease burden. This would be pay for performance, in which performance is measured in terms of impact in reducing the number of patients with a specified disease or improving quality of life. Furthermore, if a cheaper alternative to pharmaceuticals were available, such as improved sanitation compared with treatment of water-borne diseases, companies could adopt this alternative approach and still qualify for the reward. It would be necessary, of course, to make the purchasing commitment sufficiently credible to provide companies an incentive for research and development. Creating incentives based on disease burden rather than market

appeal would shift the focus of drug research from the concerns of the rich to real health improvements. Furthermore, rewarding pharmaceutical companies for a product's actual clinical effectiveness entrusts them with flexibility in choosing the best therapeutic approach and encourages them to ensure appropriate use by clinicians.

Pay for performance has many of the same negatives as the buy-out and government procurement systems. There will be difficulties in estimating the monetary value. Politicization is somewhat harder than with the government contract system, but transparency in the value used for a disability-adjusted life year may present political difficulties. Moreover, in the United States, this strategy can be followed only by moving to a single-payer system or by permitting health maintenance organizations to collaborate in ways that may pose a risk of price collusion. Finally, such a system emphasizes overall benefits, working against development of drugs for treatment of rare diseases that dramatically affect few people yet have a minimal impact on overall disease burden.

**Increasing Incentives for Orphan Drug Markets.** Using the incentive of added market exclusivity, the Orphan Drug Act encourages drug development for small markets.<sup>18,44</sup> Although there is sometimes question about their novelty, the act has generated more than 217 new products as of 2001.<sup>18,44</sup> Might such incentives be expanded to encourage industry away from the blockbuster drugs toward other health needs? The Clinton Administration proposed tax breaks for the development of products for developing nations.<sup>45</sup> Alternatively, there might be "roaming exclusivity," ie, granting firms longer periods of exclusivity on a blockbuster drug in return for developing new products of substantial developing-nation value.<sup>46</sup> An exclusivity arrangement has no direct budget implications, but tax breaks reduce government revenues, which are more difficult to enact.

These approaches would not necessarily reduce costs, except by lower-

ing the approval costs of products intended for a very narrow community of patients. After all, it is necessary for the research costs to be recovered from a relatively small group of patients. Indeed, there has been serious criticism of the prices charged for some orphan drugs, which have run as high as \$100 000 per year.<sup>46</sup> Unfortunately, with the clear exception of Roche,<sup>47</sup> the major pharmaceutical companies have generally left the orphan market to biotechnology firms.

For developing countries the cost can be spread much more widely, but someone must still pay. Roaming exclusivity, for example, is likely to produce a strong reaction against higher prices for extended periods from the patients and insurers in developed countries using the drug with the extended exclusivity. For drugs focused on diseases of developing countries, this approach is a serious competitor to government buy-out-type approaches. However, only substantial tax breaks would significantly improve the economics of developing drugs for the developing world, but such breaks might make the difference for a drug appropriate for both tourists or the military and developing countries. A continued allocation of public funding might be required to provide an adequate market within the developing world.

**Requiring New Products to Demonstrate an Improvement.** A recent proposal would have the FDA require that trials be able to show that new products, rather than placebos, are better than already approved products.<sup>1</sup> This would force the industry to concentrate on genuinely new products rather than “me-too” products. Such clinical trials could enable purchasers to fuse their drug purchasing budgets more effectively and perhaps encourage companies to seek new targets.

This proposal would significantly increase the costs of clinical trials. Trials will have to be larger to have the statistical power to demonstrate differences between products. These costs would be passed on, substantially raising the cost for approved drugs, limit-

ing access and exacerbating the crisis in health costs.

Contrary to popular belief, investment in me-too products is not necessarily wasteful. As illustrated by the statins and the selective serotonin reuptake inhibitor antidepressants, not all drugs in the same class, even those operating by similar mechanisms, have the same therapeutic or adverse effects. That is, not all me-too drugs are really the same; frequently, small differences are medically important. Finally, me-too drugs may contribute somewhat to competition among products, reducing the costs of the products, even if they are patented.

### Recommendations for the Future

Ultimately, there is no optimal solution for addressing the problems of the patent-based pharmaceutical development process. We recommend 4 complementary policies (BOX). First, it would be unwise to require that new drugs be tested against existing products prior to FDA approval. Rigorous comparative testing of drugs is highly desirable but should only be required postapproval. One option is to make FDA approval conditional on results of at least 1 randomized trial with a comparator in the same class.

Second, tiered pricing, with the price of drugs set at marginal production cost for the poorest developing countries, is highly desirable. Obviously, strict safeguards to prevent smuggling into developed countries would be absolutely essential.

Third, a combination of targeted public-sector funding of research and buy-out should be implemented for new treatments for tuberculosis, new drugs for other diseases endemic to developing countries, and possibly new antibiotics. There should be greater funding for these other conditions.

Finally, limited experiments with some of the other policy approaches seem worthwhile. International agencies might fund a disease burden incentive system targeted at water-borne diseases. Overall, 1.1 billion people lack

access to clean water worldwide; each year diarrheal diseases, 88% of which are linked to unsafe water, kill approximately 1.8 million people, 90% of whom are children.<sup>48</sup> Progress in providing clean water has been slow, and treating diarrhea and other water-borne illnesses with antibiotics is not as cost-effective as providing clean water. A new approach in this area might let the market balance the alternatives of providing clear water, of preventing disease, and of curing disease. More broadly, a “Global Orphan Drug Act” or other tax break for new treatments for a select disease, such as tuberculosis, may help spur innovation in areas not well served by the current patent system.

While the current pharmaceutical patent system is seriously flawed, there is no magic-bullet solution. The combination of the proposed 4 approaches would have a limited but positive impact, and the experiments with the targeted public-sector purchases and disease-burden approaches would permit investigation of the efficacy of major policy changes.

**Financial Disclosures:** None reported.

**Funding/Support:** This study was funded by the National Institutes of Health.

**Role of the Sponsor:** The National Institutes of Health had no role in the design and conduct of the study; the collection, management, analysis, and interpretation of the data; or the preparation, review, or approval of the manuscript.

**Acknowledgment:** We thank Yochai Benkler, JD, Yale Law School, Thomas G. Bombelles, MA, Merck, Bruce Kuhlik, JD, Pharmaceutical Research and Manufacturers of America, Reidar Lie, MD, PhD, University of Bergen, Thomas Pogge, PhD, Australian National University and Columbia University, and F. Michael Scherer, PhD, Harvard University, for their critical comments on this article. We thank Bruce Agnew, an independent contractor, for uncompensated editorial assistance.

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