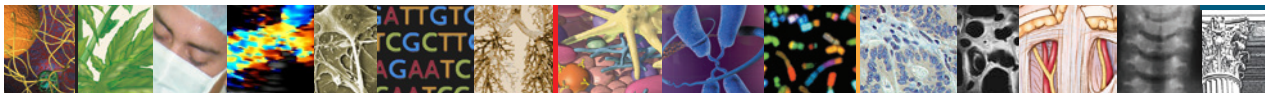

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Perspective

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State Initiatives to Control Medication Costs — Can Transparency Legislation Help?

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Spending on prescription drugs has risen sharply in the United States over the past 2 years.[1] Although thousandfold price increases for a few generic products in limited use have attracted much attention, overall spending growth has been driven more by the widespread use of costly new agents such as sofosbuvir (Sovaldi) and cumulative markups in prices of common brand-name drugs such as rosuvastatin (Crestor), imatinib (Gleevec), and etanercept (Enbrel). Coverage of these products has strained payers' budgets, forcing difficult funding choices.[2]

With little relief coming from federal legislation, states have started experimenting with their own novel approaches to the problem. One tactic has been to tighten eligibility requirements for some high-cost drugs. In the case of the hepatitis C drug sofosbuvir, for example, most state Medicaid programs have limited coverage to patients who already have liver damage. Similarly, several states have required publicly funded recipients of these drugs to abstain from drug and alcohol use. Such coverage restrictions have come under fire for being non–evidence-based and discriminatory, and particularly problematic when used to limit access to highly effective drugs.[3] In an alternative strategy, some states have contracted with nonprofit "academic detailing" organizations to assess the most current evidence about medications and educate prescribers about the relative efficacy, safety, and cost-effectiveness of their therapeutic choices.

Yet many policymakers argue that still more is needed. To evaluate the industry's controversial argument that high drug prices are a fair and necessary reflection of the high costs incurred to bring a product to market,[4] 10 states have introduced bills that would require pharmaceutical companies to disclose those research and development costs (see table). Many of these proposed laws would apply to drugs with wholesale acquisition costs of at least $10,000 per patient per year or per course of treatment, and several would mandate disclosure of profits. A few proposals would further compel reporting of prices charged in other countries as well as those paid by other purchasers in the state, including pharmacy benefits managers, which often

State Bills That Would Require Pharmaceutical Company Disclosure of Research and Development Costs.*

State	Applicable Drugs	Reporting Requirements	Status
California	New drugs with ≥$10,000 WAC per patient per year or per course of treatment Brand-name drugs for which WAC has increased more than $10,000 or 10% in the past year Generic drugs with ≥$100 WAC per month's supply for which WAC has increased more than 25% in the past year	Justification for the price or price increase Cost: marketing and acquisition	Passed senate on 6/1/16
Colorado	≥$50,000 WAC per patient per year or per course of treatment	Cost: research and development by company, predecessors, and others (e.g., state); clinical trials by company and predecessors; acquisition or licensing; manufacturing, marketing, and advertising to consumers and physicians; and patient assistance programs Price: WAC and AWP increases	Postponed on 3/10/16
New York	≥$10,000 WAC per patient per year or per course of treatment	Same as Colorado	In committee; last action 1/20/16
Oregon	≥$10,000 WAC per patient per year or per course of treatment	Same as Colorado	Failed to advance from committee
Pennsylvania	All drugs delivered for treatment in the state	Same as Colorado	In committee; last action 6/16/15
Massachusetts	Critical drugs identified on the basis of cost to public programs, cost in the state, utilization in the state, and impact of the drug on state health care cost growth	Cost: production; research and development (total, with public funds, after tax, and by others) Price: in countries selected by a state agency; paid by purchasers within state; and paid by prescription benefits managers (post rebate)	In committee; last action 5/2/16
Tennessee	Same as Massachusetts	Same as Massachusetts	In committee; last action 1/27/16
North Carolina	Brand-name antidepressants; biologics; injectables; oral cancer therapies; oral analgesics; oral medications for asthma, allergies, or other respiratory conditions; and statins	Cost: research and development; production; administration, marketing, and advertising; direct-to-consumer coupons; and financial assistance programs Price: WAC and AWP increases Profit attributable to drug (in dollars and percentage of overall profits)	In committee; last action 4/15/2015
Vermont	Up to 15 drugs for which the state spends a substantial amount and for which WAC has increased by 50% or more over the past 5 years or by 15% or more in the past year	Justification for the price increase in a format the state attorney general determines to be understandable and appropriate, which may include an explanation and percent of contribution for each factor	Signed by governor on 6/3/16
Virginia	≥$10,000 WAC per course of treatment	Cost: research and development, including clinical trials by company and others (e.g., state); manufacturing; acquisition or licensing Price: WAC and AWP increases Profit attributable to drug (in dollars and percentage of overall profits)	Delayed until 2017

* Updated as of June 3, 2016. Currently pending bills in New York, Pennsylvania, and Virginia would require third-party auditing of reports. The California, Vermont, and Virginia bills have specific language to keep proprietary information confidential. The Pennsylvania and Tennessee bills would authorize caps on excessively priced drugs. AWP denotes average wholesale price, and WAC wholesale acquisition cost.

receive substantial rebates from manufacturers that are kept secret as proprietary information. These transparency bills have been met with strenuous opposition from the pharmaceutical industry, and only Vermont's had been enacted as of June 3, 2016.

The impetus behind such efforts is the idea that the price of a drug should reflect not only an assessment of its clinical benefit — represented by its cost-effectiveness or economic value — but also the effort and resources expended in its creation. Proponents of the bills argue that payers have a right to know how a drug's current price relates to such factors as its development, manufacturing, and marketing costs and that this information would assist policymakers in determining when a price is reasonable and in pushing back when it is not.

Opponents of the bills dispute this claim, pointing out that the information that the states receive would not necessarily account for expenditures on failed products or unsuccessful research programs, which constitute a large component of the development costs of the minority of drugs that reach the market. Of course, nothing would prevent drug companies from also supplying the cost of failures, nor are states prevented from amending their bills to include such information. Proponents of transparency bills contend that even without data on the cost of failures, requiring drug-specific cost information would provide states with a better sense of what it took to create a drug than they currently possess. According to this position, reporting requirements could help identify cases in which drug development was heavily subsidized by the public through funding by the National Institutes of Health or a public–private partnership program such as the Biomedical Research and Development Authority. They could also alert state purchasers to problematic business practices engaged in by companies such as Turing or Valeant, which bought the rights to limited-source generic drugs that they played no role in developing and then sharply raised their prices.

Defining development costs is of limited value without the power to use that information to determine reimbursement — a much harder sell politically.

Advocates of transparency bills also suggest that such laws could encourage manufacturers to invest more heavily in research and development to justify the high prices of their products, in part on the basis of previous reviews that have found that only about 15% of pharmaceutical company revenues are currently invested in research and development — substantially less than is spent on marketing.[5] They add that more widespread knowledge of the cost of drug development could lead to establishment of better incentives to drive innovation to areas of high public health importance, including the development of prizes for the creation of novel antibiotics.

Laudable as these intentions are, several factors could limit the effectiveness of these measures. First, giving fair consideration to "sunk costs" that went into researching other products that did not make it to market is no easy task. Would companies be allowed to recoup the costs of a failed drug for Alzheimer's disease by raising the price of a new drug for diabetes? Should states or private-sector payers have the right to make such resource-allocation decisions?

A further question arises about the most appropriate metric for a fair assessment of a drug's selling price. If a company had a tortuous and perhaps inefficient process for developing yet another statin that was no more safe or effective than existing statins, would that justify a higher price? The economics literature would suggest that the fair price of a product should be aligned with the actual value (clinical utility) it provides, an assessment traditionally based on a drug's cost-effectiveness, rather than how much it cost to develop.

Finally, it is unclear how any required research-cost data would be verified. Of the eight state pharmaceutical transparency bills still pending, only three would require certification by third-party auditors. The prospect of multiple metrics for calculating development costs, potentially applied differently in different states, is not encouraging.

Equally important, defining development costs is of limited val-

ue without the power to use that information to determine reimbursement — a much harder sell politically and one that has not yet found much traction. Federal law, for example, mandates that state Medicaid programs cover nearly all drugs approved by the Food and Drug Administration, a requirement that undercuts states' ability to extract price concessions. Federal law also prohibits the Centers for Medicare and Medicaid Services from negotiating the prices of drugs paid for through the Medicare Part D drug-benefit programs, even though the agency uses its purchasing power to negotiate reimbursement for nearly all other medical goods and services. A few states have tried to bridge this gap by connecting transparency to reimbursement. The Tennessee proposal, for example, would authorize the state's department of health to set price caps on drugs.

An audio interview with Ameet Sarpatwari is available at NEJM.org

Transparency laws could help provide some additional information for state policymakers in a historically opaque area of the marketplace, particularly the now-hidden value of secret rebates made to prescription benefit managers, Medicaid programs, and other payers. But methodologic complexities and political realities may hamper their ability to use them to lower drug prices. In the short term, we believe that more promising policy approaches are likely to come from using currently available data to measure the relative benefits, risks, and costs of treatment alternatives and then actively disseminating those insights to prescribers, policymakers, payers, and patients. Such work is currently being done by the Institute for Clinical and Economic Review, the Drug Effectiveness Review Project, Alosa Health, the Memorial Sloan Kettering Cancer Center, the National Institute for Clinical Excellence in Britain, and the Institute for Quality and Efficiency in Health Care in Germany, among others. Ultimately, more effective use of such approaches may be the most practical weapon to enable states to drive better choices based on information that is already in the public domain.

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1. Medicine use and spending in the U.S. — a review of 2015 and outlook to 2020. IMS Institute for Healthcare Informatics, 2016 (https://www.imshealth.com/en/thought-leadership/ims-institute/reports/medicines-use-and-spending-in-the-us-a-review-of-2015-and-outlook-to-2020).
2. Barlas S. States try to control Medicaid pharmaceutical costs: numerous, diverse cost pressures force myriad reform efforts. P T 2015;40:260-2.
3. Barua S, Greenwald R, Grebely J, Dore GJ, Swan T, Taylor LE. Restrictions for Medicaid reimbursement of sofosbuvir for the treatment of hepatitis C virus infection in the United States. Ann Intern Med 2015;163:215-23.
4. Avorn J. The $2.6 billion pill — methodologic and policy considerations. N Engl J Med 2015;372:1877-9.
5. Anderson R. Pharmaceutical industry gets high on fat profits. BBC News. November 6, 2014 (http://www.bbc.com/news/business-28212223).

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Wollschlaeger v. Governor of Florida — The First Amendment, Physician Speech, and Firearm Safety

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On June 21, 2016, the full 11th Circuit Court of Appeals will hear arguments in *Wollschlaeger v. Governor of Florida*, which challenges a Florida law regulating physicians' speech related to patients' gun ownership. A decision by the court on the merits will most likely have broad implications both for states' ability to regulate physicians' speech and physicians' efforts to protect patients from firearm-related injuries, which in 2014 in the United States, included more than 33,000 deaths, most of which (21,334) were suicides.[1]

The evidence that the presence of a gun in the home increases the risk of death, especially by suicide, for all household members provides an empirical basis for the recommendation issued by several medical societies, including the American Medical Association, the American College of Physicians, and the American Academy of Pediatrics, that physicians discuss firearm safety with their patients (or in the case