Keeping a lid on drug prices

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By Jeffrey S. Flier APRIL 17, 2016

ARE DRUG PRICES too high? This recurring question is again in the news, fueled by a presidential campaign, and at least three different issues. Some highly effective new drugs, such as Sovaldi for hepatitis C, were introduced at unusually high prices; some off-patent generic drugs, like Daraprim for toxoplasmosis, had prices dramatically increased for no apparent reason; and prices of many prescription drugs seem high, especially with insured patients now paying ever higher out-of-pocket costs.

The three issues, often conflated in typically incoherent calls for action, are fundamentally different. Sovaldi is an innovative and curative therapy that saves lives and is highly cost effective despite its high price. Daraprim's exorbitant pricing reflects a clumsy, flawed generic drug regulatory system, easily gamed for short-term gain. The overall costs and reduced coverage for prescription drugs largely reflects imperfections in the way we approve and pay for drugs. To avoid policy responses that would make matters worse, we must keep these stories separate.

In a market economy, prices are set by the intersection of supply and demand, the most efficient way to incentivize production and distribution of goods and services, including drugs. There is no Platonic ideal of a "right price," determined by policy wonks and politicians. Markets for developing and

pricing drugs are hobbled by factors affecting both supply and demand, and their efficiency needs to be improved, not further undermined. When politicians recommend price controls or complex regulatory metrics to establish the “right prices,” expect an illusory short-term fix that will deprive us of tomorrow’s breakthroughs. But equally important, innovation should not be employed as a diversion to justify behaviors that drive up prices unrelated to innovation or value.

It’s worth remembering what patients want: innovative treatments to cure illness and improve lives, with prices falling over time, as now occurs when drugs go off patent. Effective new therapies are being introduced today, but often at what seem to be very high prices. Can such prices be lowered without actions that would prevent development of innovative therapies in the first place? Here are several ideas to consider.

New drugs require expensive research and development under tight regulatory oversight. The cost of developing a single new drug may exceed $2 billion when including the cost of failures. The price of a drug should reflect its value, not simply the cost of developing it. But some of the costs of development are not necessary, and if eliminated, some of the savings would be reflected in prices.

Procedures required by the FDA for development and approval of new products need to be thoroughly redesigned, including new approaches to adaptive design of clinical trials and use of biomarkers to provide earlier evidence of efficacy than available through mortality-based metrics. Thresholds to clear a drug through the FDA process should more readily consider both medical need and the severity of the disease being treated. Though many regulatory hurdles were established to guarantee safety, patients with otherwise untreatable diseases may be willing to accept an uncertain risk for the chance of benefit, and should be permitted to do so.

More important, many steps in the highly regulated development process, however originally justified, lack scientific rationale, drive up costs, and could be safely eliminated. One example: human small-scale tests of a new molecule must have the drug prepared by elaborate manufacturing procedures required for a commercial drug in full distribution, well beyond the scientific standards necessary to insure that a particular research batch of a drug is pure. This difference can literally cost millions of dollars for even the first testing, providing no significant benefit in safety. However straightforward, this reform has been difficult to achieve.

Why? The FDA often takes a flawed approach to balancing risks vs. benefits, worrying too much about risks and insufficiently about the consequences of treatments forgone because of poorly designed regulations that drive up costs.

Generics are a major way to lower prices, but there are excessively long delays for their approval. Reducing barriers to entry is a key path to lowering prices. Lower cost but highly reliable generic providers are discouraged from US markets by needlessly complex and costly procedures. If a generic drug has been approved in Germany or Canada, does it require an exhaustive additional regulatory review in the United States?

What about the payment side? Medicare, the largest payer of health care, is prohibited from using its market power to negotiate price. One reason for this is clear: If Medicare were permitted to drive drug prices to low levels, investment and innovation would be threatened. But changes in how Medicare pays for drugs and chooses those that are covered are urgently needed — the current approach is an unsustainable anachronism. One approach now being discussed is reference pricing, where a maximum payment is set for drugs with similar efficacy, with patients responsible for any charges above that. Reference pricing has been used in Europe to suppress introduction of

innovative new therapies, so a proper reference pricing system must be built from the ground up to incentivize, rather than inhibit, innovation.

We can have a world with both lower drug prices and innovation. But the necessary reforms to the regulatory and insurance systems require action within the dysfunctional political arena; when either topic is discussed in the public square, rational diagnosis and remedies are rarely heard. Biopharma makes the appropriate case for innovation, but can't avoid protecting its short-term business interests in the process. Neither political party gets it right. Democrats cast biopharma as profit-maximizing villains. Republicans reference the heavy hand of government when efforts to control Medicare drug spending is proposed. Both narratives are seriously flawed.

The antidote? To recognize that grasping for simplistic solutions is as problematic as the prices themselves. The future of the public's health depends on separating the Sovaldis from the Daraprims, the innovators from the poseurs and crooks, and the political pabulum from the complex economics of drug development, pricing, and regulation. If we do, we can eventually have both the cures we need and the prices we can afford.

Jeffrey S. Flier is the dean of Harvard Medical School.