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You Can Teach an Old Drug New Tricks

Aspirin prevents heart attacks, and good policy could give other generic drugs a second life.

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Heart disease has killed more Americans than any other condition for decades, claiming nearly a million lives a year. One of the most important advances against it came not from a new drug but a repurposed 19th-century painkiller.

Aspirin was introduced in 1899 for fevers and aches, and it quickly became one of the world's most widely used drugs. In the 1950s, a California physician, Lawrence Craven, observed that aspirin increased bleeding in his patients. He hypothesized that the drug might reduce blood clotting and thus prevent heart attacks.

Twenty years later, researchers began properly testing aspirin for this use and confirmed its effectiveness. Today, one aspirin a day reduces the risk of a second heart attack by an average of 20% for millions of Americans each year.

Aspirin's second act is a powerful example of generic drug repurposing—the discovery of new uses for long-approved, off-patent medicines. It also reveals the hurdles that prevent promising ideas from reaching patients. Without Craven to push the idea forward, aspirin's cardiovascular benefits might have gone unnoticed. Even after the insight emerged, it took years to secure the funding for clinical trials to establish aspirin's effectiveness against heart attacks.

America's system of pharmaceutical regulation treats generic-drug repurposing as serendipity rather than strategy. Once a drug's patents expire, its profit margins are too small to justify the investments required for additional private research. Even when research reveals a potential new use, adoption often remains low. This means we relegate ourselves to relying on the goodwill of physicians like Craven and piecemeal grant funding to uncover new uses and facilitate their adoption.

In 2016 metformin, long used to treat diabetes, showed promise for treating multiple sclerosis. A decade later, follow-up research remains slow and incomplete. The cholesterol drug fenofibrate is another example. In 2007, it showed encouraging evidence for slowing a common form of vision loss associated with diabetes. The results were strong enough to affect clinical practice in Australia and the U.K. Yet no one has completed the additional studies that U.S. regulators require. As a result, fenofibrate remains largely unused for that purpose in America.

There are scores of other examples, and new research by University of Chicago economist Eric Budish and coauthors suggests that a lack of stronger market incentives has left hundreds of opportunities for generic drug repurposing on the shelf.

Better policies could unlock these opportunities. The Trump administration has taken important initial steps by releasing a report that includes drug repurposing in its strategy to treat chronic diseases and save costs. The Food and Drug Administration this week began soliciting input on ways to advance drug repurposing.

The next step is to back this vision with policy changes. The first step is more public funding, structured to maximize its power to encourage generic drug repurposing. Unlike traditional grants, which pay upfront for research regardless of whether it succeeds, so-called pull funding rewards results. This is similar to how the patent system encourages the development of new drugs—companies invest knowing they will earn returns only if their drug works and is adopted. For generic repurposing, pull funding rewards researchers only when a generic drug proves it delivers benefits for a new use and is adopted for that use. That motivates researchers to work on the most promising candidates, invest in large trials capable of changing medical practice, and drive clinical adoption.

Public institutions are well-positioned to deploy pull funding. Medicare, Medicaid and other public insurers save money when low-cost drugs replace expensive treatments or prevent disease. To motivate research, the insurers could promise a portion of the realized savings to the researchers who drove the discovery. Research agencies like the National Institutes of Health could dedicate some pull funding to repurposing research.

Regulatory reform would also help. The FDA could modernize the process to ensure drug labels include all evidence-backed uses, which are key to driving adoption. Nonprofits, academics and other nontraditional developers have generated evidence for some new uses of generic drugs, but there's no clear path for them to secure label updates. The FDA could streamline routes for nonindustry developers to bring strong evidence forward and ensure drug labels include all evidence-based uses.

Breakthroughs like using aspirin against heart disease shouldn't depend solely on luck and the goodwill of an individual physician. They should be the predictable outcome of smart policy. By rewarding results and updating

regulatory pathways, we can uncover new uses for generic drugs. These long-approved, affordable medicines are on pharmacy shelves. We need the policy to unlock their full potential.

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