Development of a National Public Pharmaceutical Research and Development Institute

Health Policy Portal

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The high cost of prescription drugs has featured prominently in the 2020 presidential campaign,[1] reflecting the challenge millions of Americans face in affording their medications.[2] Of greatest concern have been routine price increases of existing brand-name drugs and rapidly escalating launch prices of novel brand-name drugs. For example, of 36 top-selling brand-name drugs available in 2012, 16 (44%) more than doubled in cost by 2019,[3] while the average annual cost of a new brand-name cancer drug now exceeds $150,000.[4]

Many of these products would not have made it to market without taxpayer-funded support. The US National Institutes of Health (NIH) alone accounts for more than half of the research and development (R&D) spend reported by major pharmaceutical companies each year.[5] This funding was linked at some level to the development of all 210 novel brand-name drugs approved between 2010 and 2016.[6] Other public entities, such the Department of Defense and state organizations like the Cancer Prevention and Research Institute of Texas,[7] also offer important support. Traditionally, funding from such institutions has covered basic and early-stage translational science, but a quarter of novel small-molecule brand-name drugs approved over the past decade were based in part on key late-stage publicly-supported contributions.[8]

To better account for these contributions, some policymakers have proposed instituting fair pricing terms on applicable drugs. In August 2019, for example, Sen. Chris Van Hollen (D-MD) and Sen. Rick Scott (R-FL), introduced the We Protect American Investment in Drugs Act (We PAID) Act, which would establish a Drug Affordability and Access Committee to determine reasonable prices for drugs with patents disclosing federal funding.[9]

Although such legislation is promising, a publicly-supported organization could be a useful supplement to help advance taxpayer-supported basic and translational science findings through to regulatory approval. This is particularly important because major pharmaceutical companies — which currently take the lead on late-stage development and regulatory approval of nearly all promising new drugs — spend less than a fifth of their revenue on R&D,[10] much less than what they do on marketing and administration.[11] Additionally, many companies "routinely distribute more than 100 percent of profits to shareholders, generating the extra cash by reducing reserves, selling off assets, taking debt, or laying off employees."[12]

Pressing health needs, meanwhile, remain unmet. A focus on short-term profits has shifted drug development away from certain areas of unmet medical need or public health importance, such as cardiovascular disease.[13] In 2019, for example, Amgen joined several other pharmaceutical companies in cutting their R&D portfolios for central nervous system drugs.[14] This January, the World Health Organization (WHO)

About This Column

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The Journal of Law, Medicine & Ethics, 47 (2019): 225-227. © 2019 The Author(s)
DOI: 10.1177/1073110520917023

sounded an alarm regarding the insufficiency of the pharmaceutical pipeline to tackle antimicrobial resistance.[15] Of 50 antibiotics in clinical testing, only 2 were for multi-drug resistant gram-negative bacteria — the biggest infectious disease threat.[16]

A national public pharmaceutical R&D institute for full-cycle drug development could help fill some of these gaps. This institute could be based at NIH, benefitting from close collaboration with existing institutes and their increasing involvement in early-phase clinical trials, and would focus on developing drugs of societal need, starting with discrete areas of market failure, such as antibiotics. To ensure that the institute delivers on its promise, its founding statutes could include a commitment to contributing to safe, adequate, and accessible supply of essential medicines in the US; to maximum transparency; and to management in the public interest. The institute's inventions could be patented — to protect against private companies that might patent public inventions and raise prices — but maintained in a patent pool subject to a "copyleft"-type license ensuring their free use. Finally, whether the institute contracted with public, nonprofit, or private manufacturers, it would be required to retain rights in the drugs it develops, and to reinvest revenues from licensing or sales into R&D.[17] The institute would therefore not only return revenues to public balance sheets and ensure broad access to newly developed drugs, but would also substantially advance open and collaborative science by ensuring greater access to the data associated with its inventions and the clinical trials undertaken, allowing for greater learning from the failures or unexpected outcomes that inevitably occur as part of the R&D process.

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Importantly, establishment of national public pharmaceutical initiative would not be without challenges. It would require a large up-front investment by the federal government. Yet this expenditure would be offset by long-term savings from reduced drug prices and avoided health care costs. Generic prices for just one commonly used medication could reduce government expenditures by hundreds of millions of dollars. For example, the US Department of Health and Human services estimated that in 2016 alone, Medicare Part D could have saved $577 million through full generic substitution of prescriptions for the heartburn drug esomeprazole (Nexium).[18]

The institute would also face a steep learning curve. However, there are good reasons to believe this challenge could be met. The US public sector has a long tradition of path-breaking innovation. For example, the Defense Advanced Research Projects Agency within the Department of Defense was instrumental in overseeing R&D that resulted in technological breakthroughs such as the Internet, Global Positioning System (GPS), and microchips.[19] Close collaboration and joint funding from the Central Intelligence Agency, the Department of Energy, and the National Science Foundation spurred advances like lithium-ion batteries, while NASA put men on the moon and helped build the International Space Station. This track record hints at what might be possible if public funds were invested in pharmaceutical R&D via a publicly controlled institute with a clear mission to deliver high-quality medications that improve or extend lives of patients across disease groups.

Note

A prior version of this Commentary was posted online at The Next System Project (https://thenextsystem.org/learn/stories/national-pharmaceutical-research-and-development-institute).

Dr. Sarpatwari and Dr. Kesselheim receive support from Arnold Ventures, the Harvard-MIT Center for Regulatory Science, and the Open Society Foundations. Their work is also funded in part by a Novo Nordisk Foundation grant for a scientifically independent Collaborative Research Programme (grant NNF17SA0027784). Dr. Brown reports grants from Open Society Foundations during the conduct of the study.

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