THE HILL

Congress should support a new strategy for approving lifesaving drugs

BY BARTLEY J. MADDEN AND ALEX TABARROK | MAY 9, 2023

Better pharmaceuticals are a bright spot in American health care.

Cancer death rates, for instance, dropped by 27 percent from 2001 to 2020, thanks not just to reduced smoking rates but also to improved treatments including vaccines that can eliminate some cervical and liver cancers from ever occurring.

Operation Warp Speed, the highly successful public-private partnership between the U.S. government and vaccine manufacturers, accelerated both COVID-19 vaccine innovations and the capacity to manufacture vaccines, used innovative shorter clinical trials and delivered (especially for the elderly) life-saving treatments.

A sign in front of the Food and Drug Administration building is seen on Dec. 10, 2020, in Silver Spring, Md. Expedited drug approvals slowed in 2022, as the FDA's controversial accelerated pathway came under new scrutiny from Congress, government watchdogs and some of the agency's own leaders. With less than a month remaining in the year, the agency's drug center has granted 10 accelerated approvals — fewer than the tally in each of the last five years, when use of the program reached all-time highs. (AP Photo/Manuel Balce Ceneta)

The Promising Pathway Act, sponsored by Sens. Mike Braun (R-Ind.) and Kristen Gillibrand (D-N.Y.), aims to build on these successes by granting the FDA a new mechanism. Provisional approval of new treatments would enable patients to access promising therapies more quickly and would sharply lower development costs, boost competition among drug developers and incentivize innovation to the ultimate benefit of patients.

Here's how the current version of the bill works: A new drug could secure provisional approval for

serious or life-threatening health conditions via early-stage clinical investigations indicating that the new drug's safety and efficacy compare favorably to approved drugs.

Importantly, provisional approval requires establishing patient registries for all such treatments and is not simply a matter of faster approval. Third-party, independent entities would manage these registries, tracking safety and effectiveness.

In order to speed up the use of this new knowledge, the de-identified, disaggregated databases would be accessible to approved researchers, medical professionals for public health research and biopharmaceutical industry researchers. Drug sponsors or the government would fund the registries, and the FDA would submit an annual report to Congress on provisionally approved drugs.

By implementing patient registries, the Promising Pathway Act aims to address some of the concerns raised by critics regarding the safety and effectiveness of a modernized, less bureaucratic system of testing and approval. These registries would provide ongoing monitoring and generate invaluable real-world data on the performance of provisionally approved drugs, thereby enabling informed decisions about their use. This information could improve treatment protocols as well as guide FDA decision-making for full approval or withdrawal of provisional approval status.

Registry data would promote transparency, foster collaboration and drive further innovation in the pharmaceutical industry. The annual report to Congress would also ensure accountability and ongoing oversight of the provisional approval process.

Patient registries would provide feedback to the biopharmaceutical industry, helping them to improve their research and development decision-making and fostering long-term innovation. Meanwhile, patients, guided by their doctors, can make informed personalized risk-reward assessments, potentially resulting in life-changing medical treatments.

Together, ideas like these could kick start a new pro-innovation paradigm with reduced developmental drug costs and heightened competition as more companies gain provisional approval. These are the ingredients for lower drug prices.

Moreover, the most effective way to ensure safety is to innovate and produce superior drugs. The Promising Pathway Act presents a thoughtful, balanced approach that expedites drug development while simultaneously creating patient registries to accelerate the production of new knowledge thereby benefiting current patients and future generations.

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