HEARTLAND DAILY NEWS

Congress Considers Warp Speed Path for New Drugs

Kevin Stone

October 25th, 2021

Legislators from both sides of the political fence have reintroduced a bill that aims to get promising drugs into the hands of patients faster.

Originally introduced in 2020, The Promising Pathway Act (PPA) would streamline the provisional approval process for drugs that have been shown to be safe but have not completed years of efficacy trials required by the U.S. Food and Drug Administration (FDA).

Four Democrats and three Republicans are sponsoring the Act, and Sen. Mike Braun (R – IN) has introduced a companion bill in the Senate.

According to the text of the bill, the process applies to drugs meant to treat "a serious or life-threatening disease or condition for which there is a reasonable likelihood that premature death will occur without early medical intervention for an individual contracting or being diagnosed with such disease or condition; or a disease or condition that poses a threat of epidemic or pandemic."

Insurers would not be allowed to decline reimbursement for drugs covered by the Act on the basis that they are "experimental".

Lower Cost, Greater Access

According to co-sponsor Rep. Bruce Westerman (R-AR), PPA would increase competition in the prescription drug market and subsequently lower costs.

"The Promising Pathways Act would allow pharmaceutical companies to petition the FDA for provisional approval if the drug has cleared early-stage clinical trials, already having proven safety," Westerman stated on his website. "The company could then sell their drug at a market acceptable rate, give patients access to innovative treatments and compete with large, monopolistic pharmaceuticals to lower consumer cost."

Rep. Mike Gallagher (R-WI), another sponsor, says the bill will improve access to drugs that could prolong or save lives.

"This bill prioritizes patients' lives, promotes innovation, and cuts through the bureaucratic red tape that can slow down the approval process," said Gallagher on his website.

In an opinion article in the Washington Times on October 2, Bart Madden, the author of *Free to Choose Medicine: Better Drugs Sooner at Lower Cost and Value Creation Principles*, and Siri Terjesen, a professor and associate dean of research at Florida Atlantic University write PPA could wipe years off the current regulatory approval process.

"Under PPA, new drugs will receive provisional approval five to seven years earlier than the status quo via a two-year provisional approval. Drugs that demonstrate patient benefits could be renewed for a maximum of six years, and the FDA could grant full approval at any time based on real-world as opposed to clinical trial data document favorable treatments results."

Cost Savings Questionable

Jeffrey A. Singer, M.D, a surgeon, and a senior fellow at the Cato Institute, says he agrees the bill will help people get access to potentially life-saving drugs sooner but questions whether PPA will save ultimately save them money.

"It is important to note that patients can and should still be allowed to not take the new medication depending upon their individual risk-benefit assessment," Singer told *Health Care News*. "I am not sure the savings in regulatory costs will be passed on to consumers because there are so many other regulatory costs as well as other moving parts that contribute to the high cost of prescription drugs, not the least of which is the third-party payer system."

While not addressing potential direct cost savings related to a reduced regulatory burden, John C. Goodman, the president of The Goodman Institute and co-publisher of *Health Care News* pointed to the relative cost-effectiveness of drug therapies compared to other medical treatments.

"Over-regulation of drugs has kept many useful drugs off the market for too long – to the detriment of patients," said Goodman. "Of all the major therapies, drugs are giving us the best return. They are more cost-effective than doctor therapy or hospital therapy. Deregulation is long overdue."

Safety Assurance

Project ALS, an advocacy group for ALS patients, points out a provision in the bill that could assure patient safety.

"Critically, the bill also establishes a requirement of a patient registry for all provisionally approved drugs," said the group in a statement. "This will ensure, first, that in order to stay approved, each ALS drug must show robust, long-term safety and efficacy. Second, this registry will provide invaluable real-world data about ALS to the broader scientific community."

PPA was referred to the House Committee on Energy and Commerce on June 8, 2021, and is awaiting further action. GovTrack gives the bill a three percent chance of being enacted.