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Step Therapy's Balancing Act — Protecting Patients while Addressing High Drug Prices

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In the absence of systematic drug-pricing reform in the United States, insurers frequently turn to utilization-management strategies to contain their rising prescription-drug expenditures. In response, state and federal policymakers have renewed their interest in legislative and regulatory actions targeting one of these strategies: step therapy. The debate regarding appropriate uses of step therapy reflects a tension between two important policy goals: safeguarding patients' access to high-quality care and constraining spending on prescription drugs, including by limiting the overuse of costly medications with uncertain efficacy. In part because policymakers have failed to address the problem of high drug prices, controlling spending often falls to insurers, who have a business interest in keeping expenditures down.

Step therapy is a utilization-management strategy whereby insurers implement tiered treatment pathways for various conditions. Patients (and their physicians) who seek approval for restricted therapies must document unsuccessful attempts at treatment with less expensive therapies in earlier "steps." Most employer-sponsored insurance plans incorporate requirements for step therapy into their drug formularies.[1,2]

By design, such utilization-management strategies create administrative burdens aimed at steering patients and physicians to an insurer's preferred treatments. Learning the rules of step therapy and complying with documentation requirements involve time and effort. Step therapy controls costs by requiring patients to try cheaper treatments before more expensive ones. Evidence suggests that such protocols reduce spending for targeted therapies, although the implications for total costs of care are uncertain. Step therapy may also promote improved quality of care when it's used to steer patients to evidence-based care, which it can do when the steps are modeled on consensus treatment guidelines. As the approach is currently used, however, its contribution to improved quality appears to be limited: a recent study examining commercial insurers' use of step therapy for specialty drugs found that only about one third of protocols aligned with clinical guidelines.[2]

Administrative burdens may be warranted when they discourage the use of treatments with low efficacy. Quality and affordability are priorities shared by patients, policymakers, and payers. But administrative burdens generate their own costly bureaucratic systems and can be difficult for patients to navigate; the burdens introduced by step therapy may hinder

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access to necessary care. For example, patients may need to obtain medical records from a previous provider and deliver them to their current physician, or they may receive a claim denial or bill that requires them to work with their insurer and the prescriber to identify and correct errors.

Recent legislative and regulatory developments at the federal and state levels illustrate these dynamics. According to one analysis, 29 states have passed laws requiring insurers to include various exceptions in step-therapy protocols.[3] Components of these laws range from procedural efforts to establish appeals processes to substantive limitations on using step therapy in cases in which a patient has already undergone an unsuccessful attempt at treatment with a required drug or the required drug is contraindicated.[3] These laws cover only a small fraction of patients in any particular state. Preemption under the federal Employee Retirement Income Security Act of 1974 (ERISA) limits states' ability to regulate self-insured employer plans, including their imposition of these kinds of requirements. Similarly, state legislation doesn't apply to patients enrolled in Medicare or other federal insurance programs.

To respond to these issues, a bipartisan group of members of both houses of Congress developed the Safe Step Act (first introduced in 2019 and reintroduced in 2021),[4] which would amend ERISA to require self-insured employer plans to allow exemptions from step-therapy protocols for several reasons. The act would require the creation of a "clear and transparent process" for beneficiaries to request exemptions, and it outlines the procedure that clinicians or patients must follow and how quickly insurers would need to approve or deny requests.

The Safe Step Act also lists five specific circumstances under which insurers would be required to grant an exception to step-therapy protocols. It would require exceptions in cases in which the required treatments have previously been ineffective for the patient, delay of effective treatment would lead to "severe or irreversible consequences," required treatments are contraindicated, required treatments would prevent the patient from performing activities of daily living, or the patient's condition is stable on the existing medication and the patient has previously received coverage approval for it. The act would also empower the executive branch to identify other circumstances that might require exemptions. Most of these exceptions, however, could create additional administrative burdens for patients and clinicians that might pose challenges for maintaining continuity of care (see table).

These legislative efforts contrast with regulatory changes made by the Trump administration, which expanded the use of step therapy in Medicare, including by allowing Medicare Advantage plans to adopt such requirements for physician-administered drugs. This expansion was presented as "delivering on [the administration's] promise to lower drug prices for patients" by enabling Medicare Advantage plans to "use private-sector tools to drive down the cost of expensive drugs."[5] The policy change included patient safeguards, primarily the restriction of step-therapy requirements to new prescriptions.

The broader question of when step therapy should be used is a nuanced one. One core problem with the way the approach is currently employed is the conflation of cost and

value. There may be strong arguments for step-therapy protocols that limit the use of high-cost, low-value medications, particularly when policymakers haven't enacted drug-pricing reforms covering these products. But protocols that limit the use of high-cost, high-efficacy medications when similarly effective, inexpensive options don't exist are more troubling.

The clinical and ethical implications of step-therapy requirements vary by context.[1] For example, this approach would probably be appropriate in the case of lipid-lowering agents: a relatively new class of drugs, PCSK9 inhibitors, are effective but costly; statins are cheap, safe, and effective, which makes them a reasonable first-line agent for most people. More concerning is the application of step therapy to new antiviral treatments for hepatitis C.[2] Although expensive, these drugs offer a safe, effective cure for a serious infectious disease, and there are no similarly effective alternatives; their value to patients and to society argues against substantial access restrictions.

State and federal legislative proposals map only weakly onto these considerations, however. The Safe Step Act and state-level proposals include basic protections for patients in cases in which harm can be foreseen (e.g., when a drug is contraindicated), but none of them consider the role of cost and effectiveness in prescription-drug coverage. These policies wouldn't alleviate the administrative complexity surrounding step therapy, nor would they protect patients from protocols requiring them to undergo unsuccessful treatment with a drug that is known to be less safe and effective than another treatment. The proposals also wouldn't limit insurers' ability to adopt step-therapy protocols that are more stringent than corresponding clinical guidelines.

Payers have turned to step-therapy protocols because we have failed to address high drug prices at a societal level, instead transferring the problem to the point of care and imposing administrative burdens on physicians and patients. Step therapy is just the latest example of this phenomenon to attract attention from policymakers. Instead of improving access to effective therapies by implementing systematic drug-pricing reform, the Safe Step Act focuses on legal action at the individual-prescription level, failing to bring down drug prices while increasing administrative costs.

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Required Exceptions to Medication Step-Therapy Protocols in the Safe Step Act.

Required Step-Therapy Exception	Opportunities	Challenges
Required treatments have previously been ineffective.	It protects patients from unnecessary, ineffective treatments or delays in receiving effective treatment. It reduces wasteful use of resources.	It's unclear what threshold the law envisions for an "ineffective" treatment or which stakeholder is empowered to determine efficacy. Documenting a history of ineffective treatment may be onerous for patients, especially if they have since switched insurance plans or providers.
Delay of effective treatment would lead to "severe or irreversible consequences," and treatment otherwise required under the protocol is reasonably expected to be ineffective.	It protects patients from harmful treatment delays.	The law doesn't define "effective treatment" or "severe or irreversible consequences" or define which stakeholder is empowered to make this determination. Documenting potential harms of delays may itself introduce delays.
Required treatments are contraindicated.	It promotes patient safety. Drug labeling may offer an established standard for "contraindication."	There is a potential for burdensome documentation of contraindications by physicians and patients.
Required treatments would prevent the patient from performing occupational responsibilities or activities of daily living, such as eating, toileting, or grooming.	It promotes patients' quality of life.	The law doesn't propose how to operationally define or measure this standard or propose which stakeholder is empowered to do so. The law doesn't consider how to interpret this standard if all treatment options may attenuate functional status or if various options may affect different activities of daily living. Documenting functional status may be burdensome. The potential for subjectivity in assessment criteria could lead to gaming and in turn elicit escalation of administrative burdens associated with approval.
The patient's condition is stable on an existing medication, and the patient has previously received approval for coverage of the medication by an insurer.	It promotes adherence and continuity of treatment. It reduces the administrative burden associated with securing duplicative approval. The prescription associated with ongoing treatment could be used as evidence of meeting this standard.	There is a risk of increased administrative burden if additional documentation is required despite the presence of a current prescription.
The executive branch may identify other circumstances that may require exemptions.	It allows flexibility to address unforeseen or new patient access challenges.	Changes are subject to political preferences and interest-group lobbying efforts.