

STEP THERAPY PATIENT PROTECTIONS



NORD®
National Organization
for Rare Disorders

NORD is working to reform step therapy protocols at the state and federal level to protect rare disease patients.

While 90% of rare diseases have no US Food and Drug Administration (FDA)-approved treatment, some rare disease patients are fortunate to have an FDA-approved medication intended to treat their rare disorder or other co-morbidities. However, improper use of “step-therapy” protocols can make timely access to these medications a challenge.

Step therapy protocols require patients to take one or more alternative medications before they can access the medicine prescribed by their provider. While this is done by insurers as a means to control high drug costs, step therapy has been increasingly applied with insufficient regard for a patient’s medical situation or treatment history.

When used improperly, step therapy protocols can delay necessary treatment and lead to adverse reactions that ultimately increase health care costs, not lower them. For example, patients changing insurance plans may be required to go off a successful treatment and take a less effective medicine, simply because it is less expensive. For rare disease patients, the use of step therapy protocols is particularly concerning as it can take years to find a diagnosis and treatment that works.

As the use of step therapy has increased, so has the need to ensure that these protocols do not interfere with appropriate care for patients. It is important to note that, in seeking to reform step therapy protocols, NORD does not advocate for ending the practice entirely. Instead, the goal of these reforms is to increase protections for patients, while enabling health plans to achieve the cost-saving benefits of step therapy, when appropriate.

FEDERAL LEGISLATION: SAFE STEP ACT

The Safe Step Act (H.R. 2163/S.464) is a bill in Congress that would protect patients with federally regulated health plans from harmful step therapy protocols. This would help the more than 150 million people with federally regulated insurance access needed medication in a timely manner.

Reference: KFF 2019 Data

CRITICAL STEP THERAPY PATIENT PROTECTIONS

Examples of important step therapy protections include:

1. Ensure step therapy protocols are based on medical criteria and clinical guidelines developed by independent experts;
2. Create a simple and accessible exceptions process for providers and patients to challenge the use of step therapy;
3. Establish a clear and expedited timeline for decisions for both emergency and non-emergency situations, to ensure patients do not experience delays in treatment; and
4. Establish a basic framework for when it is most appropriate to exempt patients from step therapy.

It's estimated that

25-30
MILLION AMERICANS

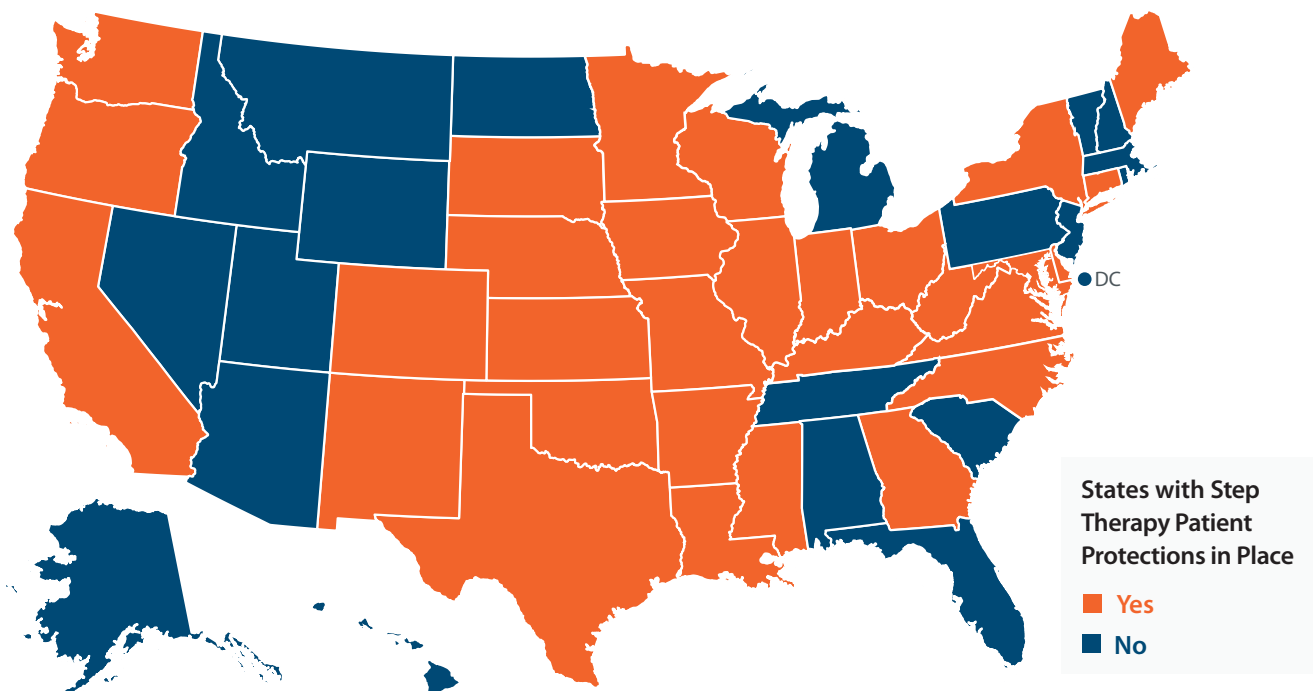
(almost 1 in 10) have rare diseases



Alone we are rare. Together we are strong.®

rareaction.org
rarediseases.org

STATES THAT HAVE ENACTED STEP THERAPY PATIENT PROTECTIONS (AS OF APRIL 2021)



Step therapy patient protocols may vary widely by state and type of insurance coverage. For the latest in your state or plan, please confirm the details of your plan with your insurance provider and physician. Learn more here: steptherapy.com/step-therapy-legislation-by-state

WHAT IS AN AUTOMATIC EXCEPTION?

"Automatic exceptions" are an important form of protection for patients in which step therapy protocols are inappropriate, and therefore immediately overridden. Some examples include when the alternative prescription drug:

- is contraindicated or will likely cause an adverse reaction
- is not in the best interest of the patient based on medical necessity
- the prescription drug is expected to be ineffective based on known clinical characteristics
- has been tried and failed by a patient while on a previous health insurance plan

WHAT ARE THE MEDICAL GUIDELINES AND CLINICAL CRITERIA?

When insurers develop step therapy protocols, they base these decisions on what are known as medical guidelines and clinical criteria. Medical guidelines and clinical criteria can be derived from a variety of sources (abstracts, written guidelines). For example, medical guidelines might specify a specific sequence that drugs are supposed to be taken in. To ensure that these protocols work for patients, it is critical that they be reviewed by independent experts who have expertise in the condition(s).

WHAT IS AN EXCEPTION PROCESS?

An "exception process" is used when the patient and/or prescribing practitioner believe step therapy is inappropriate and should therefore not be required in a particular situation.

NORD: Fighting for the rare community every day for nearly 40 years.

NORD, a 501(c)(3) organization, is a patient advocacy organization dedicated to individuals with rare diseases and the organizations that serve them. NORD, along with its more than 300 patient organization members, is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.

The mission of NORD's Rare Action Network® (RAN) is to connect and empower a unified network of individuals and organizations with the tools, training, and resources to become effective advocates for rare diseases through national and state based initiatives across the United States. To join RAN, go to rareaction.org.

For more information on NORD's step therapy policy work or to share your step therapy experience, please contact policy@rarediseases.org