



Step Therapy Reform: Hematological Conditions

Overview of hematological conditions

Hematological conditions, including blood cell cancers like leukemia or lymphoma, anemia, conditions related to HIV, sickle cell disease, and rare genetic disorders, affect millions of Americans.¹ Symptoms vary by condition, but can commonly include unexplained fatigue and weight loss, as well as more dangerous symptoms like frequent infections, difficulty forming blood clots, or unexplained bruising or bleeding.² Treatment can require blood transfusions, dietary changes, surgery, medication, or chemotherapy.³

What is step therapy?

Step therapy policies, also referred to as “fail first,” require insured individuals to try and fail on alternative treatments, sometimes with adverse effects, before the insurer or pharmacy benefit manager will cover the prescribed treatment.

Problems with step therapy

Step therapy policies can be unethical and inconsistent with standards of care, resulting in interference with the practitioner-patient relationship and significant delays in access to prescribed treatments.⁴

Step therapy requirements can be especially burdensome for patients who need highly individualized care. For them, trying and failing on alternative treatments may not be in their best interest.⁵ This could be due to lack of efficacy, lack of therapeutic equivalence, the patient’s preference regarding the medication and its associated side effects, or clinical characteristics unique to the patient that necessitate one treatment over another.⁶ Yet, the time that the patient spends trying and failing on the alternative treatment could cause the patient’s condition to progress or relapse due to the treatment’s ineffectiveness.⁷

How does step therapy negatively impact patients with hematological conditions?

Step therapy often limits timely access to commonly prescribed, evidence-based treatments within the hematology field. Delays in appropriate patient care can be particularly harmful for patients with hematological conditions.⁸ For example, Patients with sickle-cell disease may be forced to endure severe

¹ <https://www.niddk.nih.gov/about-niddk/research-areas/hematologic-diseases#:~:text=Hematologic%20diseases%2C%20disorders%20of%20the,complications%20from%20chemotherapy%20or%20transfusions.>

² <https://www.medicalnewstoday.com/articles/322260#red-blood-cell-disorders>

³ <https://www.medicalnewstoday.com/articles/322260#red-blood-cell-disorders>

⁴ <https://aimedalliance.org/wp-content/uploads/2018/10/Aimed-Alliance-Primary-Care-Survey-Report.pdf>

⁵ <https://aimedalliance.org/wp-content/uploads/2018/10/Aimed-Alliance-Primary-Care-Survey-Report.pdf>

⁶ <https://aimedalliance.org/wp-content/uploads/2018/10/Aimed-Alliance-Step-Therapy-in-Medicare-Advantage-Plans-Fact-Sheet.pdf>

⁷ <https://aimedalliance.org/wp-content/uploads/2018/10/Aimed-Alliance-Step-Therapy-in-Medicare-Advantage-Plans-Fact-Sheet.pdf>

⁸ <https://www.hematology.org/advocacy/policy-news/2019/access-to-care-and-drug-pricing-an-update-for-2019>

acute and chronic daily pain while stepping through less effective medications.⁹ Delays in treatments that are most appropriate for patients can even lead to potentially fatal outcomes. Individuals with Waldenstrom's macroglobulinemia, for instance, can have a stroke due to the increase of proteins in their blood that the physician-preferred drug could have prevented.¹⁰

Patient story

Dorothy was diagnosed with Chronic Myelogenous Leukemia. Her insurance company's step therapy requirement forced her to start treatment with a traditional chemotherapy medication.¹¹ Only after Dorothy failed on the medication for nine and a half months, showing very little positive change in her treatment and resulting in significant side effects, did the insurance company finally approve the use of a newer, less toxic, and more effective treatment. The newer medication was the one preferred by Dorothy's physician from the start. Dorothy's condition immediately improved and she did not experience the same side effects.

⁹ <https://www.hematology.org/advocacy/policy-news/2019/access-to-care-and-drug-pricing-an-update-for-2019>

¹⁰ <https://www.hematology.org/advocacy/policy-news/2019/access-to-care-and-drug-pricing-an-update-for-2019>

¹¹ https://www.ils.org/sites/default/files/file_assets/f-73-14-7403884_zgsZT3R3_MDSB622StepTherapyTestimony.pdf